

GERON CORP
Form 10-K
March 07, 2019
UNITED STATES

SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

FORM 10 K

(Mark One)

ANNUAL REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT OF 1934
For the Fiscal Year Ended December 31, 2018

or

TRANSITION REPORT PURSUANT TO SECTION 13 OR 15(d) OF THE SECURITIES EXCHANGE ACT
OF 1934

For the transition period from to .

Commission File Number: 0 20859

GERON CORPORATION

(Exact name of registrant as specified in its charter)

Delaware	75 2287752
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
149 Commonwealth Drive, Suite 2070, Menlo Park, CA	94025
(Address of principal executive offices)	(Zip Code)

Registrant's telephone number, including area code: (650) 473 7700

Securities registered pursuant to Section 12(b) of the Act:

Title of each class	Name of each exchange on which registered
Common Stock, \$0.001 par value	The Nasdaq Stock Market LLC

Securities registered pursuant to Section 12(g) of the Act: None

Indicate by check mark if the registrant is a well known seasoned issuer, as defined in Rule 405 of the Securities Act. Yes No

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Indicate by check mark if the registrant is not required to file reports pursuant to Section 13 or Section 15(d) of the Act. Yes No

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes No

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S T (§232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes No

Indicate by check mark if disclosure of delinquent filers pursuant to Item 405 of Regulation S K (§229.405 of this chapter) is not contained herein, and will not be contained, to the best of registrant's knowledge, in definitive proxy or information statements incorporated by reference in Part III of this Form 10 K or any amendment to this Form 10 K.

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non accelerated filer, a smaller reporting company, or emerging growth company. See the definitions of "large accelerated filer," "accelerated filer," "smaller reporting company," and "emerging growth company" in Rule 12b 2 of the Exchange Act.

Large accelerated filer	Accelerated filer
Non-accelerated filer	Smaller reporting company
	Emerging growth company

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act.

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b 2 of the Act). Yes No

The aggregate market value of voting and non voting common equity held by non affiliates of the registrant was approximately \$623,076,000 based upon the closing price of the registrant's common stock on June 29, 2018 on the Nasdaq Global Select Market. The calculation of the aggregate market value of voting and non voting common equity held by non affiliates of the registrant excludes shares of common stock held by each officer, director and stockholder that the registrant concluded were affiliates on that date. This determination of affiliate status is not necessarily a conclusive determination for other purposes.

As of March 1, 2019, there were 186,392,682 shares of common stock outstanding.

DOCUMENTS INCORPORATED BY REFERENCE:

Document	Form 10 K Parts
Portions of the Registrant's definitive proxy statement for the 2019 annual meeting of stockholders to be filed pursuant to Regulation 14A within 120 days of the Registrant's fiscal year ended December 31, 2018	III

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In this report, unless otherwise indicated or the context otherwise requires, “Geron,” “the registrant,” “we,” “us,” and “our” refer to Geron Corporation, a Delaware corporation.

Forward Looking Statements

This annual report on Form 10-K, including “Business” in Part I, Item 1 and “Management’s Discussion and Analysis of Financial Condition and Results of Operations” in Part II, Item 7, contains forward looking statements that involve risks and uncertainties, as well as assumptions that, if they never materialize or prove incorrect, could cause the results of Geron Corporation, or Geron or the Company, to differ materially from those expressed or implied by such forward looking statements. All statements other than statements of historical fact are statements that could be deemed forward looking statements. In some cases, forward looking statements can be identified by the use of terminology such as “may,” “expects,” “plans,” “intends,” “will,” “should,” “projects,” “believes,” “predicts,” “anticipates,” “estimates,” “potential” or the negative thereof or other comparable terminology. The risks and uncertainties referred to above include, without limitation, risks related to our ability to timely transition the imetelstat program from Janssen Biotech, Inc., or Janssen, to us, uncertainty of non-clinical and clinical trial results or regulatory approvals or clearances, the future development of imetelstat, including any future efficacy or safety results that may cause the benefit risk profile of imetelstat to become unacceptable, our need for additional capital to support the development and commercialization of imetelstat and to otherwise grow our business, enforcement of our patent and proprietary rights, potential competition and other risks that are described herein and that are otherwise described from time to time in our Securities and Exchange Commission reports including, but not limited to, the factors described in Part I, Item 1A, “Risk Factors,” of this annual report on Form 10-K. Geron assumes no obligation for and except as required by law, disclaims any obligation to update these forward looking statements to reflect future information, events or circumstances.

Calculation of Aggregate Market Value of Non-Affiliate Shares

For purposes of calculating the aggregate market value of shares of our common stock held by non-affiliates as set forth on the cover page of this annual report on Form 10-K, we have assumed that all outstanding shares are held by non-affiliates, except for shares held by each of our executive officers, directors and 5% or greater stockholders. In the case of 5% or greater stockholders, we have not deemed such stockholders to be affiliates unless there are facts and circumstances which would indicate that such stockholders exercise any control over our Company. These assumptions should not be deemed to constitute an admission that all executive officers, directors and 5% or greater stockholders are, in fact, affiliates of our Company, or that there are no other persons who may be deemed to be affiliates of our Company. Further information concerning shareholdings of our executive officers, directors and principal stockholders is incorporated by reference in Part III, Item 12 of this annual report on Form 10-K.

PART I

ITEM 1. BUSINESS

Company Overview

We are a late-stage clinical biopharmaceutical company that is focused on the development and commercialization of innovative therapeutics for hematologic myeloid malignancies. We have global rights to imetelstat, a first-in-class telomerase inhibitor, that was discovered and developed at Geron. We believe clinical data from two Phase 2 clinical trials of imetelstat (IMerge and IMbark, discussed below) conducted by Janssen Biotech, Inc., or Janssen, support further development of imetelstat in hematologic myeloid malignancies. We are working with Janssen to transition the imetelstat program to us. See further discussion below regarding our past and current relationship with Janssen.

We plan to open patient screening and enrollment by mid-year of 2019 in a Phase 3 clinical trial (Part 2 of IMerge) to evaluate imetelstat in transfusion dependent patients with Low or Intermediate-1 risk myelodysplastic syndromes, or MDS, who have relapsed after or are refractory to prior treatment with an erythropoiesis stimulating agent, or ESA, have not received prior treatment with either a hypomethylating agent or lenalidomide and do not have a deletion 5q chromosomal abnormality. This target population of lower risk MDS patients depend on serial red blood cell transfusions to manage symptoms of anemia and fatigue. However, dependency on transfusions is associated with poor survival, because of toxicity due to iron overload, as well as potential infections and allergic reactions. The ultimate goal for most trials of investigational agents in lower risk MDS is to enable patients to become transfusion independent for as long as possible. In December 2018, we reported results from the Phase 2 portion of IMerge in which 37% of patients experienced red blood cell transfusion independence for at least 8 consecutive weeks, or an 8-week RBC-TI, rate. Importantly, this 8-week RBC-TI rate was observed in patients with high transfusion burdens, an indicator of a more difficult to treat population. Patients enrolled into the Phase 2 portion of IMerge had a baseline median red blood cell transfusion burden of eight units per eight weeks with a range of four to 14 units. Our results compare favorably to currently used treatments in a similar patient population, such as hypomethylating agents, or HMAs, which have a reported 8-week RBC-TI rate of 17%, or lenalidomide, which has a reported 8-week RBC-TI rate of 27%. In addition, among the patients in the Phase 2 portion of IMerge who achieved a durable response, as reflected by achieving a 24-week RBC-TI, all showed a hemoglobin rise of ≥ 3.0 g/dL compared to baseline during the transfusion-free interval. We believe these data suggest potential disease-modifying activity of imetelstat treatment.

Regarding our myelofibrosis, or MF, program, we reported data in December 2018 from the IMbark Phase 2 clinical trial, including the median overall survival of 29.9 months observed in the trial in comparison to the median overall survival of 14 – 16 months for patients previously treated with janus kinase, or JAK, inhibitors. We plan to discuss the IMbark data with experts in MF, as well as regulatory authorities, to consider how these results compare with other therapies currently available to MF patients, and to gain a better understanding of the potential significance of these results to patients and physicians. Because IMbark is the first clinical trial to apply rigorous, objective eligibility criteria to define patients considered relapsed or refractory to JAK inhibitors, we believe feedback from these discussions could provide important information on the feasibility, scope and design, including possible outcome measures, of any potential future clinical trials for imetelstat in Intermediate-2 or High-risk MF patients who have relapsed after or are refractory to prior treatment with a JAK inhibitor. We expect to outline our decision whether to continue late-stage development of imetelstat in MF by the end of the third quarter of 2019. This decision will be influenced by our assessment of what would be required to achieve clinical and regulatory success in MF, including the cost and duration of any potential clinical trials.

We had approximately \$182.1 million in cash, cash equivalents, restricted cash and current and noncurrent marketable securities as of December 31, 2018, which is sufficient to commence the planned Phase 3 portion IMerge. If approved for marketing by regulatory authorities, we plan to commercialize imetelstat in the United States ourselves and seek

potential commercialization partners for territories outside of the United States.

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Telomerase: Scientific Rationale

Telomeres and Telomerase in Normal Development

In the human body, normal growth and maintenance of tissues occurs by cell division. However, most cells are only able to divide a limited number of times, and this number of divisions is regulated by telomere length. Telomeres are repetitions of a deoxyribonucleic acid, or DNA, sequence located at the ends of chromosomes. They act as protective caps to maintain stability and integrity of the chromosomes, which contain the cell's genetic material. Normally, every time a cell divides, the telomeres shorten. Eventually, they shrink to a critically short length, and as a result, the cell either dies by apoptosis or stops dividing and senesces.

Telomerase is a naturally occurring enzyme that maintains telomeres and prevents them from shortening during cell division, such as stem cells that must remain immortalized to support normal health. Telomerase consists of at least two essential components: a ribonucleic acid, or RNA, template (hTR), which binds to the telomere, and a catalytic subunit (hTERT) with reverse transcriptase activity, which adds a specific DNA sequence to the chromosome ends. The 2009 Nobel Prize for Physiology or Medicine was awarded to Drs. Elizabeth H. Blackburn, Carol W. Greider and Jack Szostak, former Geron collaborators, for the discovery of how chromosomes are protected by both telomeres and telomerase.

Telomerase is active during embryonic development, enabling the rapid cell division that supports normal growth. During the latter stages of human fetal development and in adulthood, telomerase is repressed in most cells, and telomere length gradually decreases during a lifetime. In tissues that have a high turnover throughout life, such as blood and gut, telomerase can be transiently upregulated in progenitor cells to enable controlled, self limited proliferation to replace cells lost through natural cell aging processes. As the progeny of progenitor cells mature, telomerase is downregulated and telomeres shorten with cell division, preventing uncontrolled proliferation.

Telomeres and Telomerase in Cancer

Telomerase is upregulated in many tumor progenitor cells, enabling the continued and uncontrolled proliferation of the malignant cells that drive tumor growth and progression. Telomerase expression has been found to be present in approximately 90% of biopsies taken from a broad range of human cancers. Our nonclinical studies, in which the telomerase gene was artificially introduced and expressed in normal cells grown in culture, have suggested that telomerase does not itself cause a normal cell to become malignant. Instead, the sustained upregulation of telomerase enables tumor cells to maintain telomere length, providing them with the capacity for limitless proliferation. We believe that the sustained upregulation of telomerase is critical for tumor progression as it enables malignant progenitor cells to acquire cellular immortality and avoid apoptosis, or cell death.

Telomerase Inhibition: Inducing Cancer Cell Death

We believe that inhibiting telomerase may be an attractive approach to treating cancer because it may limit the proliferative capacity of malignant cells. We and others have observed in various in vitro and rodent tumor models that inhibiting telomerase results in telomere shortening and arrests uncontrolled malignant cell proliferation and tumor growth. In vitro studies have suggested that tumor cells with short telomeres may be especially sensitive to the anti proliferative effects of inhibiting telomerase. Our nonclinical data also suggest that inhibiting telomerase is particularly effective at limiting the proliferation of malignant progenitor cells, which have high levels of telomerase and are believed to be important drivers of tumor growth and progression.

Imetelstat: The First Telomerase Inhibitor to Advance to Clinical Development

Imetelstat is a lipid conjugated 13 mer oligonucleotide that we designed to be complementary to and bind with high affinity to the RNA template of telomerase, thereby directly inhibiting telomerase activity. Imetelstat does not elicit its effect through an antisense inhibition of protein translation. The compound has a proprietary thio phosphoramidate backbone, which is designed to provide resistance to the effect of cellular nucleases, thus conferring improved stability in plasma and tissues, as well as improved binding affinity to its target. To improve the ability of imetelstat to penetrate cellular membranes, we conjugated the oligonucleotide to a lipid group. Imetelstat's IC₅₀, or half maximal inhibitory concentration, is 0.5 – 10 nM in cell free assays. Single dose kinetics in patients has shown dose dependent increases in exposure to imetelstat, with a plasma half life, which is the time it takes for the

concentration or amount of imetelstat to be reduced by half, ranging from 4 – 5 hours. Data from animal studies and clinical trials have suggested that the residence time of imetelstat in bone marrow is long, with 0.19 – 0.51 μM observed at 41 – 45 hours after a 7.5 mg/kg dose in patients. Imetelstat also has been shown in nonclinical studies to exhibit relatively preferential inhibition of the clonal proliferation of malignant progenitor cells compared to normal progenitors. For these reasons, imetelstat has been studied as a potential treatment for malignant diseases.

Imetelstat is the first telomerase inhibitor to advance to clinical development. The Phase 1 trials that we completed evaluated the safety, tolerability, pharmacokinetics and pharmacodynamic effects of imetelstat. We established doses and dosing schedules that were tolerable and achieved target exposures in patients that were consistent with those required for efficacy in animal models. Following intravenous administration of imetelstat using tolerable dosing regimens, clinically relevant and significant inhibition of telomerase activity was observed in various types of tissue in which telomerase activity is measurable, including normal bone marrow hematopoietic cells, malignant plasma cells, hair follicle cells and peripheral blood mononuclear cells. Dose limiting toxicities included thrombocytopenia, or reduced platelet count, and neutropenia, or reduced neutrophil count.

Disease Characteristics of Hematologic Malignancies

Hematologic malignancies, or blood cancers, are classified according to the predominant location of the malignancy. A hematologic myeloid malignancy is a cancer that occurs in the precursor cells to red blood cells, platelets and white blood cells, such as granulocytes. Examples include acute myelogenous leukemia, chronic myelogenous leukemia, MDS and the myeloproliferative neoplasms, such as essential thrombocythemia, or ET, polycythemia vera and MF. These are different from lymphocytic malignancies which typically occur in the lymphoid lineage that includes white blood cells, such as T lymphocytes and B lymphocytes. Examples of lymphoid malignancies include acute lymphoblastic leukemia, chronic lymphocytic leukemia, lymphomas and multiple myeloma.

Many hematologic myeloid malignancies, such as ET, MF, and MDS, have been shown to arise from malignant progenitor cells in the bone marrow that express higher telomerase activity and have shorter telomeres when compared to normal healthy cells.

Unmet Medical Need in Myelodysplastic Syndromes

MDS is a group of blood disorders in which the proliferation of malignant progenitor cell clones in the bone marrow results in disordered and ineffective production of the myeloid lineage, which includes red blood cells, white blood cells and platelets. In MDS, bone marrow and peripheral blood cells may have abnormal, or dysplastic, cell morphology. MDS is frequently characterized clinically by severe anemia, or low red blood cell counts, and low hemoglobin. In addition, other peripheral cytopenias, or low numbers of white blood cells and platelets, may cause life threatening infections and bleeding. Transformation to acute myelogenous leukemia, or AML, occurs in up to 30% of MDS cases and results in poorer overall survival.

MDS is the most common of the myeloid malignancies. There are approximately 60,000 people in the United States living with the disease and approximately 16,000 reported new cases of MDS in the United States every year. MDS is primarily a disease of the elderly, with median age at diagnosis around 70 years. The majority of patients, approximately 70%, fall into what are considered to be the lower risk groups at diagnosis, according to the International Prognostic Scoring System, or IPSS, that takes into account the presence of a number of disease factors, such as cytopenias and cytogenetics, to assign relative risk of progression to AML and overall survival.

Chronic anemia is the predominant clinical problem in patients who have lower risk MDS. Many of these patients become dependent on red blood cell transfusions due to low hemoglobin. Serial red blood cell transfusions can lead to elevated levels of iron in the blood and other tissues, which the body has no normal way to eliminate. Iron overload is

a potentially dangerous condition. Studies in patients with MDS have shown that iron overload resulting from regular red blood cell transfusions is associated with a poorer overall survival and a higher risk of developing AML.

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There have been no new drugs approved by the United States Food and Drug Administration, or FDA, for MDS therapy since 2006 and clinicians note that currently available therapies are likely to fail the majority of patients within two to three years after treatment initiation even if there is initial favorable response. Typically, patients with lower risk MDS are treated with ESAs, such as erythropoietin, or EPO. Although ESAs provide an improvement in anemia in approximately 50% of patients, the effect is transient with a median duration of treatment of approximately two years. Once ESAs fail for patients, HMAs and lenalidomide have been used to improve anemia, but with limited success, such as reported 8-week RBC-TI rates of 17% for azacitidine, an HMA, and 27% for lenalidomide. No drug therapy has been shown prospectively to prolong survival in lower risk MDS, nor to delay disease progression.

Unmet Medical Need in Myelofibrosis

MF, a type of myeloproliferative neoplasm, is a chronic blood cancer in which abnormal or malignant precursor cells in the bone marrow proliferate rapidly, causing scar tissue, or fibrosis, to form. As a result, normal blood production in the bone marrow is impaired and may shift to other organs, such as the spleen and liver, which can cause them to enlarge substantially. People with MF may have abnormally low or high numbers of circulating red blood cells, white blood cells or platelets, and abnormally high numbers of immature cells in the blood or bone marrow. MF patients can also suffer from debilitating constitutional symptoms, such as drenching night sweats, fatigue, severe itching, or pruritus, abdominal pain, fever and bone pain. The estimated prevalence of MF in the United States, or U.S., is approximately 13,000 patients, with an annual incidence of approximately 3,000 patients. Up to 20% of patients with MF develop AML.

Approximately 70% of MF patients are classified as having Intermediate 2 or High-risk disease, as defined by the Dynamic International Prognostic Scoring System Plus, or DIPSS Plus, described in a 2011 Journal of Clinical Oncology article. There is currently only one targeted drug therapy, ruxolitinib, a JAK inhibitor, approved by the FDA and other regulatory authorities for treating these MF patients. Currently, no drug therapy is approved for those patients who fail or no longer respond to that treatment, and median survival for such MF patients is only approximately 14 – 16 months, representing a significant unmet medical need.

Developing Imetelstat to Treat Hematologic Myeloid Malignancies

Proof of Concept of Imetelstat's Disease Modifying Potential

We believe that imetelstat may have the potential to suppress the proliferation of malignant progenitor cell clones to allow recovery of normal hematopoiesis in patients with hematologic myeloid malignancies. Early clinical data from a Phase 2 trial of imetelstat in patients with ET, or the ET Trial, and a pilot study of imetelstat in patients with MF conducted at Mayo Clinic, or the Pilot Study, suggest imetelstat may exhibit such disease modifying activity. These data were published in two separate articles in a September 2015 issue of The New England Journal of Medicine.

Reported adverse events, or AEs, and laboratory investigations associated with imetelstat in the ET Trial and the Pilot Study included cytopenias, gastrointestinal symptoms, constitutional symptoms, and hepatic biochemistry abnormalities. Dose limiting toxicities, such as profound and prolonged thrombocytopenia and neutropenia, and other safety issues, including death, were observed in the ET Trial and the Pilot Study. In those trials, such myelosuppression was managed by dose holds and modification rules.

IMerge (Phase 2/3 Trial) in Lower Risk MDS

Trial Design

IMerge is a two-part clinical trial evaluating imetelstat in transfusion dependent patients with Low or Intermediate-1 risk, also referred to as lower risk, MDS, who have relapsed after or are refractory to prior treatment with an ESA. Part 1 of IMerge was designed as a Phase 2, open-label, single-arm trial to assess the efficacy and safety of imetelstat administered as an intravenous infusion at a starting dose of 7.5 mg/kg every four weeks in approximately 30 patients, and originally was conducted by Janssen as part of a Collaboration and License Agreement, or the Collaboration Agreement. See further discussion below regarding our past and current relationship with Janssen. The first patient was dosed in January 2016. To be eligible for the Phase 2 portion of IMerge, patients were required to be

transfusion dependent, defined as requiring at least four units of packed red blood cells, or RBCs, over an eight week period during the 16 weeks before entry into the trial.

The primary efficacy endpoint of IMerge is the rate of RBC transfusion independence, or RBC-TI, lasting at least eight weeks, defined as the proportion of patients without any RBC transfusion during any consecutive eight weeks since entry to the trial, or 8-week RBC-TI rate. Key secondary endpoints include the rate of RBC-TI lasting at least 24 weeks, or 24-week RBC-TI rate, and the rate of hematologic improvement-erythroid, or HI-E, defined as a rise in hemoglobin of at least 1.5 g/dL above the pretreatment level for at least eight weeks or a reduction of at least four units of RBC transfusions over eight weeks compared with the prior RBC transfusion burden. Other secondary efficacy endpoints include the time to and duration of RBC-TI; the proportion of patients achieving Complete Response, or CR, or Partial Response, or PR, according to the 2006 International Working Group, or IWG, criteria for MDS; the proportion of patients requiring RBC transfusions and the transfusion burden; the proportion of patients requiring the use of myeloid growth factors and the dose; assessments of the change in the patients' quality of life using several validated instruments; as well as an assessment of overall survival and time to progression to AML.

32 patients were initially enrolled in the Phase 2 portion of IMerge, of which a cohort of 13 patients had not received prior treatment with either an HMA or lenalidomide and did not have a deletion 5q chromosomal abnormality, also known as non-del(5q). Preliminary data from the Phase 2 portion of IMerge were presented at the European Hematology Association, or EHA, Annual Congress, in June 2018. These data showed that the 13-patient initial cohort exhibited an increased rate and durability of transfusion independence compared to the overall trial population (8-week RBC-TI rate: 54% vs. 34%). The safety profile in the Phase 2 portion of IMerge was consistent with prior clinical trials of imetelstat in hematologic malignancies, and no new safety signals were identified. The most frequently reported adverse events were cytopenias, which were predictable, manageable and reversible, in most cases, including Grade 3 and 4, or severe, neutropenia and thrombocytopenia. In addition, reported adverse events did not differ significantly between the overall trial population and the 13-patient initial cohort.

Based on the preliminary data from the initial cohort of 13 patients, Janssen expanded new patient enrollment in the Phase 2 portion of IMerge and enrolled 25 additional patients, or an expansion cohort, who are non-del(5q) and naïve to HMA and lenalidomide treatment, to increase the clinical experience and confirm the benefit-risk profile of imetelstat in this target patient population. In November 2017, the first patient was dosed in the expanded Phase 2 portion of IMerge and enrollment was completed in February 2018.

Detailed results for the target patient population (n=38) from the combined initial cohort of 13 patients and expansion cohort of 25 patients were recently presented at the 60th American Society of Hematology, or ASH, Annual Meeting and Exposition in December 2018. A summary of the results is below.

ASH Presentation Highlights

In the ASH presentation, results were reported using a clinical cut-off date of October 26, 2018. For the initial 13-patient cohort, the median follow-up was 29.1 months and for the 25-patient expansion cohort, the median follow-up was 8.7 months. The median number of treatment cycles was 8.0 (range: 1 – 34) and the mean dose intensity was 6.9 mg/kg/cycle. The baseline characteristics of the aggregate 38 patients in the combined cohorts highlight the high transfusion burden of these patients, indicating the significant disease burden of this patient population.

Patient Baseline Characteristics (n=38)	
Median age (range), years	71.5 (46-83)
Male, n (%)	25 (66%)
Eastern Cooperative Oncology Group (ECOG) Performance Standard 0-1, n (%)	34 (89%)
International Prognostic Scoring System risk, n (%)	
Low	24 (63%)
Intermediate-1	14 (37%)
Baseline median (range) RBC transfusion burden, units/8 weeks	8 (4–14)
WHO 2001 category, n (%)	
Refractory Anemia with Ringed Sideroblasts (RARS) or Refractory Cytopenia with Multilineage Dysplasia and Ringed Sideroblasts (RCMD-RS)	27 (71%)
All others	11 (29%)
Prior ESA use, n (%)	34 (89%)
Serum EPO > 500 mU/mL, n (%)	12 ^a (32%)

^aOf the 37 patients with sEPO (serum erythropoietin) levels reported.

The 8-week RBC-TI rate for the 38 patients in the combined cohorts was 37% and 26% of patients achieved a durable response with 24-week RBC-TI. In addition, among the patients achieving durable transfusion independence, all showed a hemoglobin rise of ≥ 3.0 g/dL compared to baseline during the transfusion-free interval. We believe these data suggest potential disease-modifying activity of imetelstat treatment. In addition, similar 8-week RBC-TI rates were observed between ringed sideroblast positive (37%) patients and other patients (36%), and between those patients with baseline erythropoietin levels >500 mU/mL (33%) and ≤ 500 mU/mL (40%), indicating the broad clinical activity of imetelstat in the Phase 2 portion of this trial. These and other efficacy data are also summarized in the table below:

Key Efficacy Outcomes	n=38
Rate of 8-week RBC-TI, n (%)	14 (37%)
Rate of 24-week RBC-TI, n (%)	10 (26%)
Median time to onset of RBC-TI (range), weeks	8.1 (0.1-33.1)
Median duration of RBC-TI (range), weeks	Not Evaluable (17.0-NE)
Rate of transfusion reduction (hematologic improvement-erythroid, or HI-E), n (%)	27 (71%)
Mean relative reduction of RBC transfusion burden from baseline, %	-68%

CR+ marrow CR + PR (per International Working Group, or IWG), n (%)	8 (21%)
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As summarized in the table below, the safety profile was consistent with prior clinical trials of imetelstat in hematologic malignancies, and no new safety signals were identified. Nineteen patients (50%) had dose reductions and 26 patients (68%) had cycle delays. Reversible Grade 3 liver function test, or LFT, elevations were observed in three patients (8%) and an independent Hepatic Review Committee deemed the observed LFT elevations were not imetelstat-related hepatic toxicities.

Most Common Treatment-Emergent Adverse Events (TEAE)	Patients > 1 TEAE	
	Grade 1-2	Grade > 3
Neutropenia	1	21
Thrombocytopenia	2	23
Anemia	2	7
Leukopenia	0	7
Aspartate Aminotransferase, or AST, increased	3	3
Alanine Aminotransferase, or ALT, increased	5	2
Headache	5	1
Bronchitis	4	2
Nasopharyngitis	6	0
Diarrhea	6	0
Peripheral edema	6	0
Back pain	4	2

The majority of Grade ≥ 3 neutropenia and thrombocytopenia were reversible within four weeks as shown in the table below:

Occurrence and Reversibility of Cytopenias	All events n=38	Recovered in < 4wks of patients with an event
Neutrophils, n (%)		
Grade 3	10 (26%)	8 (80%)
Grade 4	12 (32%)	12 (100%)
Platelets, n (%)		
Grade 3	14 (37%)	13 (93%)
Grade 4	10 (26%)	9 (90%)

Current Status of the Phase 2 Portion of IMerge

The Phase 2 portion of IMerge has been officially closed to new patient enrollment and patients remaining in the treatment phase are eligible to continue to receive imetelstat treatment, per investigator discretion. Data collection and patient follow-up continue in accordance with the trial protocol and is being conducted by Janssen during the program transition period. In connection with the transition of the imetelstat program, we expect sponsorship for IMerge to be transferred from Janssen to us by the end of the second quarter of 2019. Once the IND transfer has been completed, we will assume responsibility for treating and following patients in accordance with the Phase 2 trial protocol.

We expect more mature data from the patients continuing in the treatment phase of the Phase 2 portion of IMerge to be available in 2019 and anticipate submitting such data for presentation at a future medical conference in 2019.

Plan for IMerge Phase 3 Clinical Trial to Begin by Mid-Year 2019

Based on the results of the Phase 2 portion of IMerge, we intend to continue the development of imetelstat in lower risk MDS. Importantly, the 37% 8-week RBC-TI rate observed in the Phase 2 portion of IMerge compares favorably to currently used treatments in a similar patient population, such as HMAs, with a reported 8-week RBC-TI rate of 17% or lenalidomide, with a reported 8-week RBC-TI rate of 27%. Also, the IMerge results were observed in patients with high transfusion burdens, an indicator of a more difficult to treat population and among the patients who achieved durable transfusion independence in the Phase 2 portion of IMerge, all showed a hemoglobin rise of ≥ 3.0 g/dL compared to baseline during the transfusion-free interval. We believe these data suggest potential disease-modifying activity of imetelstat treatment.

We expect patient screening and enrollment of Part 2, or the Phase 3 portion, of IMerge, to begin by mid-year of 2019, after sponsorship of the ongoing imetelstat clinical trials has been transferred from Janssen to us. The Phase 3 portion of IMerge is a double-blind, randomized, placebo-controlled trial in approximately 170 patients, which will evaluate imetelstat in transfusion dependent patients with Low or Intermediate-1 risk MDS, who have relapsed after or are refractory to prior treatment with an ESA, have not received prior treatment with either an HMA or lenalidomide and do not have a deletion 5q chromosomal abnormality. We expect the trial to be conducted at multiple medical centers globally, including North America, Europe and Asia. Trial design information for the Phase 3 portion of IMerge, including patient eligibility criteria and locations of clinical sites, will be posted on clinicaltrials.gov.

IMbark (Phase 2 Trial) in Relapsed/Refractory MF

Trial Design

IMbark was designed as a Phase 2 clinical trial to evaluate two starting dose levels of imetelstat (either 4.7 mg/kg or 9.4 mg/kg administered by intravenous infusion every three weeks) in approximately 200 patients with Intermediate-2 or High-risk MF who have relapsed after or are refractory to prior treatment with a JAK inhibitor. We believe IMbark is the first clinical trial to rigorously define this specific patient population, as currently there is no established clinical definition for relapsed after or refractory to prior treatment with a JAK inhibitor. Patients eligible for the trial were required to have active symptoms of MF, together with worsening of splenomegaly-related abdominal pain at any time after the start of JAK inhibitor therapy, and either: no reduction in spleen volume or size after 12 weeks of JAK inhibitor therapy, or worsening splenomegaly after the start of JAK inhibitor therapy, as documented by an increase in spleen volume from its lowest point, or nadir, by 25% when measured by imaging, or an increase in spleen size when assessed by palpation. IMbark was originally conducted by Janssen as part of the Collaboration Agreement.

The co-primary efficacy endpoints for the trial are spleen response rate, defined as the proportion of patients who achieve a $\geq 35\%$ reduction in spleen volume assessed by imaging, and symptom response rate, defined as the proportion of patients who achieve a $\geq 50\%$ reduction in Total Symptom Score, or TSS, at 24 weeks. Key secondary endpoints are safety and overall survival. Other secondary efficacy endpoints include the number of patients achieving complete remission, or CR, or partial remission, or PR, clinical improvement, or CI, and anemia, spleen and symptom responses. Exploratory endpoints include cytogenetic and molecular responses, as well as leukemia free survival.

The first patient in IMbark was dosed in September 2015 and the last patient was enrolled in October 2016. Janssen initiated a protocol-specified primary analysis of IMbark in the second quarter of 2018. The IMbark protocol-specified primary analysis, which included an assessment of overall survival, or OS, coincided with the protocol-specified final analysis for the trial, due to an overlap in the dates triggering each analysis, which resulted in a joint primary/final analysis, which we refer to herein as the primary analysis. The results of this primary analysis and updated data on overall survival were presented at the ASH Annual Meeting and Exposition in December 2018. A summary of the results is below.

ASH Presentation Highlights

As reported in the ASH presentation, a total of 107 patients were enrolled in IMbark (48 in the 4.7 mg/kg dosing arm and 59 in the 9.4 mg/kg dosing arm). At the time of the April 26, 2018 clinical cut-off for the primary analysis, patients in IMbark had been followed for a median of 22.6 months (range: 0.2 – 27.4), including median treatment duration of 26.9 weeks (range: 0.2 – 118.1). Seven patients remained on active treatment and 50 patients were being followed for survival. The baseline characteristics of the patients enrolled in IMbark, as presented at ASH and highlighted below, indicate the advanced nature of the disease in, and the potential difficulty to treat, this patient population:

Patient Baseline Characteristic Highlights	4.7 mg/kg (n = 48)	9.4 mg/kg (n = 59)	Total (n = 107)
Median age (range), years	68.5 (44 – 84)	67 (31 – 86)	68.0 (31 – 86)
Myelofibrosis subtype, n (%)			
Primary	27 (56%)	36 (61%)	63 (59%)
Post-Essential Thrombocythemia, or ET	9 (19%)	10 (17%)	19 (18%)
Post-Polycythemia Vera, or PV	12 (25%)	13 (22%)	25 (23%)
DIPSS risk status, n (%)			
Intermediate-1 risk	1 ^a (2%)	0 (0%)	1 (1%)
Intermediate-2 risk	28 (58%)	34 (58%)	62 (58%)
High-Risk	19 (40%)	25 (42%)	44 (41%)
Spleen volume (MRI) – Median, IRC (range), cm ³	3353 (726 – 7243)	2998 (890 – 7607)	3167 (726 – 7607)
Platelet count – Median (range), x10 ⁹ /L	153 (74 – 1097)	146 (65 – 798)	147 (65 – 1097)
Time since initial diagnosis – Median (range), months	49 (2 – 227)	43 (7 – 201)	44 (2 – 227)
Duration of prior JAKi Tx – Median (range), months	22 (3 – 90)	25 (1 – 73)	23 (1 – 90)
Triple negative ^b , n (%)	10 (21%)	16 (28%)	26 (25%)
High molecular risk ^c , n (%)	36 (75%)	35 (61%)	71 (68%)

^aIndicated in electronic case report form comments, but does not appear in statistical output. This is a protocol deviation.

^bAbsence of JAK2 V617F, CALR or MPL mutations. Indicator of a poor prognosis.

^cOne or more mutations in ASXL1, EZH2, SRSF2, IDH1, or IDH2. An indicator of progressive disease in the patient. Six patients (10%) in the 9.4 mg/kg dosing arm and no patients in the 4.7 mg/kg dosing arm had a spleen response per imaging. The spleen volume response rate observed, including in the 9.4 mg/kg dosing arm, was less than that reported in clinical trials with JAK inhibitors in front-line MF patients. Nineteen patients (32%) in the 9.4 mg/kg dosing arm and three patients (6%) in the 4.7 mg/kg dosing arm had a symptom response.

For the assessment of OS, the clinical cut-off date for the ASH presentation was October 22, 2018. The median follow-up was 27.4 months (range: 0.2 – 33.0). The median OS in the 9.4 mg/kg dosing arm was 29.9 months. These and other efficacy data are also summarized in the table below:

	Dosing Arm	
n (%)	4.7 mg/kg	9.4 mg/kg
Number of enrolled patients	48	59
Spleen response rate	0 (0%)	6 (10%)
Symptom response rate	3 (6%)	19 (32%)
Complete remission rate	0 (0%)	0 (0%)
Partial remission rate	0 (0%)	1 (2%)
Median overall survival	19.9 mos	29.9 mos

As summarized in the table below, the safety profile was consistent with prior clinical trials of imetelstat in hematologic malignancies, and no new safety signals were identified.

n (%)	4.7 mg/kg (n = 48)		9.4 mg/kg (n = 59)	
	All Grades	Grade ≥ 3	All Grades	Grade ≥ 3
Hematologic (≥ 10% in either arm)				
Thrombocytopenia	11 (23%)	11 (23%)	29 (49%)	24 (41%)
Anemia	15 (31%)	15 (31%)	26 (44%)	23 (39%)
Neutropenia	5 (10%)	5 (10%)	21 (36%)	19 (32%)
Leukopenia	3 (6%)	3 (6%)	8 (14%)	8 (14%)
Non-hematologic (≥ 20% in either arm)				
Nausea	15 (31%)	1 (2%)	20 (34%)	2 (3%)
Vomiting	10 (21%)	1 (2%)	8 (14%)	1 (2%)
Diarrhea	18 (38%)	2 (4%)	18 (31%)	0 (0%)
Fatigue	10 (21%)	3 (6%)	16 (27%)	4 (7%)
Cough	11 (23%)	0 (0%)	9 (15%)	0 (0%)
Dyspnea	9 (19%)	6 (13%)	14 (24%)	3 (5%)
Abdominal pain	10 (21%)	2 (4%)	14 (24%)	3 (5%)
Asthenia	9 (19%)	3 (6%)	14 (24%)	6 (10%)
Pyrexia	8 (17%)	1 (2%)	13 (22%)	3 (5%)
Edema peripheral	13 (27%)	0 (0%)	11 (19%)	0 (0%)

Most cytopenias resolved within four weeks. Grade 3/4 LFT elevations were observed in seven patients on study. An independent Hepatic Review Committee deemed that the observed LFT elevations were not imetelstat-related hepatic toxicities.

Current Status of IMbark

The trial has been officially closed to new patient enrollment since March 2018 and has entered an extension phase to enable patients remaining in the treatment phase to continue to receive imetelstat treatment, per investigator discretion. During the extension phase, which is being conducted by Janssen during the program transition period, standard data collection will primarily consist of safety information.

In connection with the transition of the imetelstat program, we expect sponsorship for IMbark to be transferred from Janssen to us by the end of the second quarter of 2019. Once the IND transfer has been completed, we will be responsible for following patients in accordance with the extension phase protocol.

For MF, we plan to discuss the results of the IMbark primary analysis, including the assessment of OS, with experts in MF, as well as regulatory authorities, to consider how these results compare with other therapies currently available to MF patients, and to gain a better understanding of the potential significance of these results to patients and physicians. We believe feedback from these discussions will provide important information on the feasibility, scope and design, including possible outcome measures, of any potential future clinical trials for imetelstat in Intermediate-2 or High-risk MF patients who have relapsed after or are refractory to prior treatment with a JAK inhibitor. We expect to outline our decision whether to continue late-stage development of imetelstat in MF by the end of the third quarter of 2019. This decision will be influenced by our assessment of what would be required to achieve clinical and regulatory success in MF, including the cost and duration of any potential clinical trials.

Transition from Janssen

In December 2014, we entered into the Collaboration Agreement with Janssen, pursuant to which Janssen conducted IMbark and IMerge. Janssen terminated the Collaboration Agreement effective September 28, 2018, and upon the effective date of termination, we regained the global rights to the imetelstat program. Under the terms of the Collaboration Agreement, Janssen is required to provide operational support for the imetelstat program during transition of the program to us. Each company is responsible for its own costs incurred related to transition activities, unless otherwise specified in the Collaboration Agreement. We expect the transition process to be completed by September 2019 to enable the orderly transfer of all ongoing clinical, regulatory, medical affairs and non-clinical activities to us, including transfer of the sponsorship of ongoing imetelstat clinical trials from Janssen to us. In addition, following the effective date of termination of the Collaboration Agreement, we expect Janssen to supply imetelstat to us for up to 24 months during a transition period for clinical manufacturing and such supply will be charged to us at Janssen's cost plus a premium. See "Licensing—Former Collaboration and License Agreement with Janssen" below, for more information about the Collaboration Agreement.

We have engaged Parexel International (IRL) Limited, or Parexel, a global contract research organization, or CRO, to support imetelstat clinical development activities. In addition to recently hiring a head of Pharmacovigilance and Drug Safety and a Chief Medical Officer in January of 2019, we are actively recruiting senior personnel to staff our internal drug development group, as well as contract with subject matter experts in clinical science, biostatistics, clinical operations, pharmacovigilance, quality, manufacturing and regulatory affairs.

Intellectual Property

Intellectual property, including patent protection, is very important to our business. We file patent applications in the United States and other jurisdictions, and we also rely on trade secret protection and contractual arrangements to protect aspects of our business. An enforceable patent with appropriate claim coverage can provide an advantage over competitors who may seek to employ similar approaches to develop therapeutics, and so the future commercial success of imetelstat, and therefore our future success, will be in part dependent on our intellectual property strategy. The information provided in this section should be reviewed in the context of the section entitled "Risks Related to Protecting Our Intellectual Property" under Item 1A, "Risk Factors".

The development of biotechnology products, including imetelstat, typically includes the early development of a technology, followed by rounds of increasingly focused innovation around a product opportunity, including identification and definition of a specific product candidate and uses thereof, manufacturing processes, product formulation and administration methods. The result of this process is that biotechnology products are often protected by several families of patent filings that are filed at different times during product development and cover different aspects of the product. Consequently, earlier filed, broad technology patents will usually expire ahead of patents covering later developments, such as product formulations, so that patent expirations on a product may span several years. Patent coverage may also vary from country to country based on the scope of available patent protection. There are also opportunities to obtain an extension of patent coverage for a product in certain countries, which adds further complexity to the determination of patent life.

We endeavor to monitor worldwide patent filings by third parties that are relevant to our business. Based on this monitoring, we may determine that an action is appropriate to protect our business interests. Such actions may include negotiating patent licenses where appropriate, filing oppositions against a patent, filing a request for post grant review against a patent or filing a request for the declaration of an interference with a patent application or issued patent.

Imetelstat

We own issued patents in the United States, Europe and other countries related to imetelstat. Composition of matter patents generally provide the most material coverage, and therefore may convey competitive advantages. Because imetelstat is still under development, subsequent innovation and associated patent filings may provide additional patent coverage with later expiration dates. Examination of overseas patent applications typically lags behind U.S. examination particularly where cases are filed first in the United States. It may be possible to obtain patent term extensions of some patents in some countries for claims covering imetelstat which could further extend the patent term.

Product Candidate	U.S. Patent Status / Europe Patent Status / Japan Patent Status /		
	Expiration Date	Expiration Date	Expiration Date
Imetelstat (composition of matter)	Issued / 2025	Issued / 2024	Issued / 2024

Our patent rights relating to imetelstat include those covering composition claims to the drug molecule and related nucleic acid telomerase inhibiting molecules, as well as reagents useful in manufacturing processes for the drug, and method of treatment and kit claims, certain of which are co-owned with other entities.

Upon the effective date of termination of the Collaboration Agreement with Janssen on September 28, 2018, we regained all of the worldwide rights to imetelstat. In accordance with the termination provisions of the Collaboration Agreement, we have an exclusive worldwide license for intellectual property developed under the Collaboration Agreement for the further development of imetelstat, without any economic obligations to Janssen with respect to such license. Janssen has assigned to us certain intellectual property developed by it under the Collaboration Agreement. We now bear all of the costs for maintaining, prosecuting and litigating all imetelstat intellectual property that we own.

Telomerase

Our U.S. patent rights relating to telomerase that cover technologies, such as variants of the protein component of human telomerase, or hTERT, are co-owned with and in-licensed exclusively from the University of Colorado. We expect the last of these U.S. patent rights to expire in 2019. A U.S. patent for identifying inhibitors of telomerase activity is in-licensed from the University of Texas Southwestern Medical Center and the University of California and will expire in 2019. The expiration of these patents is not expected to have any impact on our intellectual property rights related to imetelstat, or our continued planned development of imetelstat. See Item 1A, "Risk Factors" for additional information regarding our patent rights relating to telomerase.

Licensing

Former Collaboration and License Agreement with Janssen

On November 13, 2014, we entered into the Collaboration Agreement, pursuant to which we granted to Janssen the exclusive rights to develop and commercialize imetelstat worldwide for all indications in oncology, including hematologic myeloid malignancies, and all other human therapeutic uses. The Collaboration Agreement became effective on December 15, 2014, and we received \$35 million from Janssen as an upfront payment.

We regained the global rights to imetelstat upon Janssen's termination of the Collaboration Agreement effective September 28, 2018. As a result of the termination of the Collaboration Agreement, we will not receive any further milestone payments or royalties from Janssen for the development or commercialization of imetelstat, including any clinical development or sales milestones, and Janssen has no further obligations to us or any third parties, such as clinical sites or vendors, to fund any of the ongoing or any potential future imetelstat clinical trials.

Under the termination provisions of the Collaboration Agreement, Janssen is required to provide certain operational support for the imetelstat program during transition of the program to us. The transition process is expected to occur through September 2019 to enable the orderly transfer of all ongoing clinical, regulatory, medical affairs and non-clinical activities to us, including the transfer of the sponsorship of ongoing imetelstat clinical trials from Janssen to us. Each company is responsible for costs incurred related to transition activities unless otherwise specified in the

Collaboration Agreement. Under the Collaboration Agreement, Janssen is required, among other things, to:

- assign all regulatory files and regulatory clearances specific to imetelstat to us, including sponsorship of the ongoing clinical trials, IMbark and the Phase 2 portion of IMerge;
- transfer all safety data to us;
- facilitate negotiations between us and any subcontractors of Janssen performing development or manufacturing activities related to imetelstat;
- transfer any remaining inventory of imetelstat to us at Janssen's cost plus a premium, and use commercially reasonable efforts to facilitate an orderly and prompt transition of manufacturing activities to us; and

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supply imetelstat drug product to us at Janssen's cost plus a premium for up to 24 months following the termination of the Collaboration Agreement, while we seek to re-establish our own supply chain for clinical manufacturing of imetelstat.

Until the sponsorship responsibilities for imetelstat transfers from Janssen to us, including the U.S. Investigational New Drug, or IND, application and all foreign regulatory applications, Janssen will continue conducting IMbark and the Phase 2 portion of IMerge. Patients currently enrolled in IMbark and the Phase 2 portion of IMerge will continue to receive treatment and follow-up under the respective trial protocols. After September 28, 2018, the effective termination date of the Collaboration Agreement, our responsibility for imetelstat development costs, including ongoing conduct of the extension phase of IMbark and the Phase 2 portion of IMerge, and costs for the prosecution of patents that were licensed to Janssen under the Collaboration Agreement increased from 50% to 100%. In the second quarter of 2018, Janssen informed us that no patients remain on study or in follow-up in the Pilot Study. Therefore, we expect Janssen to close the Pilot Study, and the related IND under which the Pilot Study has been conducted will be inactivated.

For a further discussion of the Collaboration Agreement, see Note 4 on License Agreements in Notes to Financial Statements of this Form 10-K. Information about the transition of the imetelstat program from Janssen to us should be reviewed in the context of the section entitled "Risks Related to Transition of the Imetelstat Program from Janssen to Geron" included in Item 1A, "Risk Factors" of this Form 10-K.

Other License Agreements

In addition to the above agreement, we have also granted licenses to a number of other organizations in the ordinary course of our business to utilize aspects of our technologies to develop and commercialize products outside of the imetelstat program. These include:

- a license to Janssen Pharmaceuticals, Inc., or Janssen Pharmaceuticals, an affiliate of Janssen, for the research, development and commercialization of products based on specialized oligonucleotide backbone chemistry and novel amidates for disorders, excluding cancers originating from the blood or bone marrow. In connection with this license, we also granted to Janssen Pharmaceuticals a non-exclusive worldwide license under our patent rights covering the synthesis of monomers, which are the building blocks of oligonucleotides;
- two licenses, both of which will expire in 2019, to companies to use or commercialize telomerase immortalized cells in drug discovery research;
- six licenses, five of which will expire in 2019, to companies to develop and commercialize reagent kits, or to provide services, for the measurement of telomere length or telomerase activity for research purposes;
- a license to a company to develop and commercialize a particular telomerase-based technology for cancer detection; and
- a license to a company for the development of cancer immunotherapies for veterinary applications.

See Item 7, "Management's Discussion and Analysis of Financial Condition and Results of Operations—Revenues" for a further discussion of revenues from our license agreements. We expect revenues under our license agreements related to our telomerase technology to be eliminated by the end of 2019 due to upcoming patent expirations on such technology.

Concentration of Revenues

Our revenues were \$1.1 million, \$1.1 million and \$6.2 million for the years ended December 31, 2018, 2017 and 2016, respectively. See Item 7, "Management's Discussion and Analysis of Financial Condition and Results of Operations—Revenues" for additional detail regarding the composition of our revenues.

Manufacturing

A typical sequence of steps in the manufacture of imetelstat drug product includes the following key components:

- starting materials, which are well defined raw materials that are used to make bulk drug substance;

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• bulk drug substance, which is the active pharmaceutical ingredient in a drug product that provides pharmacological activity or other direct effect in the treatment of disease; and

• final drug product, which is the finished dosage form that contains the drug substance that is shipped to the clinic for patient treatment.

In accordance with the termination provisions of the Collaboration Agreement, Janssen is required to supply imetelstat drug product to us at Janssen's cost plus a premium for up to 24 months following the termination of the Collaboration Agreement, while we seek to re-establish our own supply chain for clinical manufacturing of imetelstat. During the transition of the imetelstat program from Janssen to us, we plan to engage third party contractors to perform certain process development and other technical and scientific work with respect to imetelstat, as well as supply starting materials and manufacture drug substance and drug product. Many of these contractors previously had relationships with Geron related to the manufacture and/or supply of imetelstat.

We do not have direct control over third party personnel or operations. These third party contractors, and/or any other contractors that we may rely upon for the manufacture and/or supply of imetelstat, typically complete their services on a proposal by proposal basis under master supply agreements and may need to make substantial investments to enable sufficient capacity increases and cost reductions, and to implement those regulatory and compliance standards necessary for successful Phase 3 clinical trials and commercial production. These third party contractors, and/or any other contractors that we may rely upon for the manufacture and/or supply of imetelstat, may not be able to achieve such capacity increases, cost reductions, or regulatory and compliance standards, and even if they do, such achievements may not be at a commercially reasonable cost. We are responsible for establishing any long term commitments or commercial supply agreements with any of the third party contractors for imetelstat. The information provided in this section should be reviewed in the context of the section entitled "Risks Related to Manufacturing" and "Risks Related to Transition of the Imetelstat Program from Janssen to Geron" under Item 1A, "Risk Factors".

Consultants

To rebuild our drug development expertise, we have established, and expect to continue to establish, consulting agreements with drug development professionals, clinicians and regulatory experts with experience in numerous fields, including clinical science, biostatistics, clinical operations, pharmacovigilance, quality, manufacturing and regulatory affairs. We retain each consultant according to the terms of a consulting agreement. Under such agreements, we pay them a consulting fee and reimburse them for out of pocket expenses incurred in performing their services for us. In addition, we have in the past and may again in the future grant options to purchase our common stock to consultants, subject to the vesting requirements contained in the consulting agreements. Our consultants may be employed by other entities and therefore may have commitments to their employer, or may have other consulting or advisory agreements that may limit their availability to us.

Competition

The pharmaceutical and biotechnology industries are intensely competitive. Other pharmaceutical and biotechnology companies and research organizations currently engage in or have in the past engaged in efforts related to the biological mechanisms related to imetelstat, the study of telomeres, telomerase, or our proprietary oligonucleotide chemistry, and the research and development of therapies for the treatment of hematologic myeloid malignancies. In addition, other products and therapies that could directly compete with imetelstat currently exist or are being developed by pharmaceutical and biopharmaceutical companies and by academic institutions, government agencies and other public and private research organizations.

If approved for commercial sale for the treatment of lower risk MDS, imetelstat would compete against a number of treatment options, including erythropoiesis stimulating agents and other hematopoietic growth factors; immunomodulators, such as lenalidomide by Celgene Corporation, or Celgene.; hypomethylating agents, such as

azacitidine by Celgene and decitabine by Janssen; in addition to investigational treatments that may be further along in development than imetelstat, such as oral versions of azacitidine; histone deacetylase inhibitors; TGF-beta superfamily inhibitors, such as luspatercept by Acceleron Pharma, Inc., or Acceleron, in collaboration with Celgene; PI3 Kinase inhibitors; proteasome inhibitors; aminopeptidase inhibitors, such as tosedostat by CTI Biopharma

Corporation, or CTI Biopharma; TLR2-specific antibodies; TPO agonists, such as romiplostim by Amgen Inc.; anti-CD33 antibodies; anti-CD38 antibodies, such as daratumumab by Genmab A/S in collaboration with Janssen; anti-CD123 antibodies, such as talacotuzumab by Janssen; antagonists of Toll-like receptor signaling; retinoic acid receptor alpha agonists, such as SY-1425 by Syros Pharmaceuticals; hypoxia-inducible factor prolyl hydroxylase inhibitors, such as roxadustat by FibroGen, Inc.; Fas ligand inhibitors; immune checkpoint regulators; and JAK-STAT pathway inhibitors.

If approved for commercial sale for the treatment of MF, imetelstat would compete against Incyte Corporation's ruxolitinib, or Jakafi®, which is orally administered. In clinical trials, Jakafi® reduced spleen size, abdominal discomfort, early satiety, bone pain, night sweats and itching in MF patients. Recently, there have also been reports of overall survival benefit as well as improvement in bone marrow fibrosis from Jakafi® treatment. Other treatment modalities for MF include hydroxyurea for the management of splenomegaly, leukocytosis, thrombocytosis and constitutional symptoms; splenectomy and splenic irradiation for the management of splenomegaly and co-existing cytopenias, or low blood cell counts; chemotherapy and pegylated interferon. Drugs for the treatment of MF-associated anemia include erythropoiesis stimulating agents, androgens, danazol, corticosteroids, thalidomide and lenalidomide. There are other investigational treatments for MF further along in development than imetelstat, such as pacritinib by CTI Biopharma, momelotinib by Sierra Oncology, and fedratinib by Celgene, which have reported results from Phase 3 clinical trials. Other investigational treatments for MF include inhibitors of the JAK-STAT pathway, such as NS-018 by NS Pharma, Inc.; histone deacetylase inhibitors; interleukin-3 receptor targeted agents; inhibitors of heat shock protein 90; hypomethylating agents; PI3 Kinase and mTOR inhibitors; anti-fibrosis antibodies, such as PRM-151 from Promedior, Inc.; hedgehog and SMO inhibitors; PIM kinase inhibitors; IAP inhibitors; anti-LOX2 inhibitors; recombinant pentraxin 2 protein; KIP-1 activators; TGF-beta superfamily inhibitors, such as sotatercept and luspatercept by Acceleron, in collaboration with Celgene; FLT inhibitors; BET inhibitors, such as CPI-0610 by Constellation Pharmaceuticals, Inc.; SMAC mimetics, such as LCL161 by Novartis Pharmaceuticals Corporation; and tyrosine kinase inhibitors.

Smaller companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies. We anticipate increased competition in the future as new companies explore treatments for hematologic myeloid malignancies, which may significantly impact the commercial viability of imetelstat. Academic institutions, government agencies and other public and private research organizations may also conduct research, seek patent protection and establish collaborative arrangements for research, clinical development and marketing of products similar to imetelstat. These companies and institutions compete with us in recruiting and retaining qualified development and management personnel as well as in acquiring technologies complementary to the imetelstat program.

In addition to the above factors, imetelstat will face competition based on:

- product efficacy and safety;
- convenience of product administration;
- cost of manufacturing;
- the timing and scope of regulatory consents;
- status of coverage and reimbursement;
- price; and
- patent position, including potentially dominant patent positions of others.

As a result of the foregoing, competitors may develop more commercially desirable or affordable products than imetelstat, or achieve earlier patent protection or product commercialization than we may be able to achieve with imetelstat. Competitors have developed, or are in the process of developing, technologies that are, or in the future may be, competitive to imetelstat. Some of these products may have an entirely different approach or means of accomplishing therapeutic effects similar or superior to those that may be demonstrated by imetelstat. Competitors

may develop products that are safer, more effective, or less costly than imetelstat, or more convenient to administer to patients and, therefore, present a serious competitive threat to imetelstat. In addition, competitors may price their

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products below what we may determine to be an acceptable price for imetelstat, may receive better third-party payor coverage and/or reimbursement, or may be more cost-effective than imetelstat. Such competitive products or activities by competitors may render imetelstat obsolete, which may cause us to cease any further development or future commercialization of imetelstat, which would severely and adversely affect our financial results, business and business prospects, and the future of imetelstat, and might cause us to cease operations.

Government Regulation

Regulation by governmental authorities in the United States and other countries is a significant factor in the development, manufacture and marketing of imetelstat. Imetelstat will require regulatory approval by governmental agencies prior to commercialization. In particular, potential human therapeutic products, such as imetelstat, are subject to rigorous preclinical and clinical testing and other approval procedures of the FDA and similar regulatory authorities in European and other countries. Various governmental statutes and regulations also govern or influence testing, manufacturing, safety, labeling, storage, import, export, distribution and recordkeeping related to such products and their marketing. The process of obtaining these approvals and the subsequent compliance with appropriate statutes and regulations require the expenditure of substantial time and money, and there can be no guarantee that approvals will be granted. The information provided in this section should be reviewed in the context of the sections entitled “Risks Related to the Development of Imetelstat” and “Risks Related to Regulatory Approval and Commercialization of Imetelstat” under Item 1A, “Risk Factors”.

United States Food and Drug Administration Regulatory Approval Process

Prior to commencement of clinical trials involving humans, preclinical testing of new pharmaceutical products is generally conducted on animals in the laboratory to evaluate the potential efficacy and safety of a product candidate. The results of these trials are submitted to the FDA as part of an Investigational New Drug, or IND, application, which must become effective before clinical testing in humans can begin. The FDA can place an IND on clinical hold at any time, which prevents the conduct of clinical trials under the IND until safety concerns are addressed by the IND sponsor to the FDA’s satisfaction. Typically, clinical evaluation involves a time consuming and costly three phase trial process. In Phase 1, clinical trials are conducted with a small number of healthy volunteers or patients afflicted with a specific disease to assess safety and to evaluate the pattern of drug distribution and metabolism within the body. In Phase 2, clinical trials are conducted with groups of patients afflicted with a specific disease in order to determine preliminary efficacy, optimal dosages and expanded evidence of safety. The Phase 2 trials can be conducted comparing the investigational treatment to a comparator arm, or not. If used, a comparator usually includes standard of care therapy. Safety and efficacy data from Phase 2 clinical trials, even if favorable, may not provide sufficient rationale for proceeding to a Phase 3 clinical trial. In Phase 3, large scale, multi center, comparative trials are conducted with patients afflicted with a target disease to provide sufficient data to demonstrate the efficacy and safety required by the FDA. The FDA closely monitors the progress of each of the three phases of clinical testing and may, at its discretion, re evaluate, alter, suspend, or terminate the trials. Human clinical trials must be conducted in compliance with Good Clinical Practice regulations and applicable laws, with the oversight of Institutional Review Boards for the protection of human subjects. The manufacture of drug product candidates is subject to requirements that drugs be manufactured, packaged and labeled in conformity with current Good Manufacturing Practices and applicable laws.

The results of the preclinical and clinical testing of drugs and complete manufacturing information are submitted to the FDA in the form of a New Drug Application, or NDA, for review and for approval prior to commencement of commercial sales. Submission of an NDA requires the payment of a substantial user fee to the FDA, which may be waived in certain cases. In responding to an NDA submission, the FDA may approve the drug for commercialization, impose limitations on its indications for use and labeling, including in the form of Risk Evaluation and Mitigation Strategies or may issue a complete response letter. Even if an NDA is approved, its sponsor is subject to ongoing and

pervasive regulatory compliance requirements.

European and Other Regulatory Approval Process

Prior to initiating clinical trials in a region outside of the United States, a clinical trial application must be submitted and reviewed by the appropriate regulatory authority regulating the country in which the trial will be conducted. Whether or not FDA clearance or approval has been obtained, approval of a product by comparable regulatory authorities in Europe and other countries is necessary prior to commencement of marketing the product in

such countries. The regulatory authorities in each country may impose their own requirements and may refuse to grant an approval, or may require additional data before granting it, even though the relevant product has been cleared or approved by the FDA or another authority. As with the FDA, the regulatory authorities in the European Union, or EU, and other developed countries have lengthy approval processes for pharmaceutical products. The process for gaining approval in particular countries varies, but generally follows a similar sequence to that described for FDA approval. In Europe, the European Medicine Agency, or EMA, and the European Committee for Proprietary Medicinal Products, or CPMP, provide a mechanism for EU member states to exchange information on all aspects of product licensing. The EU has established the EMA for the evaluation of medical products, with both a centralized procedure with which the marketing authorization is recognized in all EU member states and a decentralized procedure, the latter being based on the principle of licensing within one member country followed by mutual recognition by the other member countries.

Orphan Drug Designation

For a drug to qualify for orphan drug designation by the FDA, both the drug and the disease or condition must meet certain criteria specified in the Orphan Drug Act, or ODA, and FDA's implementing regulations. Orphan drug designation is granted by the FDA's Office of Orphan Drug Products in order to support development of medicines for underserved or rare diseases and patient populations that affect fewer than 200,000 people in the United States or, if the disease or condition affects more than 200,000 individuals annually in the United States, if there is no reasonable expectation that the cost of developing and making the drug would be recovered from sales in the United States. Orphan drug designation qualifies the sponsor of the drug for various development incentives of the ODA, including, if regulatory approval is received, the potential for seven years of market exclusivity with certain limited exceptions and certain tax credits for qualified clinical testing. A marketing application for a prescription drug product that has received orphan drug designation is not subject to a prescription drug user fee unless the application includes an indication for a disease or condition other than the rare disease or condition for which the drug was granted orphan drug designation. The granting of orphan drug designation does not alter the standard regulatory requirements and process for obtaining marketing approval. The safety and effectiveness of a drug must be established through adequate and well controlled studies. Orphan drug exclusivity does not prevent the FDA from approving a different drug for the same disease or condition, or the same drug for a different disease or condition.

On June 11, 2015 and December 23, 2015, the FDA granted orphan drug designation to imetelstat for the treatment of MF and MDS, respectively.

Orphan drug designation by the European Commission provides regulatory and financial incentives for companies to develop and market therapies that treat a life threatening or chronically debilitating condition affecting no more than five in 10,000 persons in the EU, and where no satisfactory treatment is available. In the EU, orphan drug designation also entitles a party to financial incentives such as reduction of fees or fee waivers, as well as protocol assistance from the EMA during the product development phase, and direct access to the centralized authorization procedure. In addition, ten years of market exclusivity is granted following drug product approval, meaning that another application for marketing authorization of a later similar medicinal product for the same therapeutic indication will generally not be approved in the EU. This period may be reduced to six years if the orphan drug designation criteria are no longer met, including where it is shown that the product is sufficiently profitable to not justify maintenance of market exclusivity.

On December 14, 2015, the EMA granted orphan drug designation to imetelstat for the treatment of MF.

Fast Track Designation

Fast Track designation provides opportunities for frequent interactions with FDA review staff, as well as eligibility for priority review, if relevant criteria are met, and rolling review. Fast Track designation is intended to facilitate and expedite development and review of a New Drug Application to address unmet medical needs in the treatment of serious or life-threatening conditions. However, Fast Track designation does not accelerate conduct of clinical trials or mean that the regulatory requirements are less stringent, nor does it ensure that imetelstat will receive marketing approval or that approval will be granted within any particular timeframe. In addition, the FDA may withdraw Fast Track designation if it believes that the designation is no longer supported by data emerging from the imetelstat clinical development program.

In October 2017, the FDA granted Fast Track designation to imetelstat for the treatment of adult patients with transfusion-dependent anemia due to Low or Intermediate-1 risk MDS who are non-del(5q) and who are refractory or resistant to treatment with an ESA.

Fraud and Abuse, Data Privacy and Security, and Transparency Laws and Regulations

We may also be subject to additional healthcare regulation and enforcement by the federal government and by authorities in the states and foreign jurisdictions in which we conduct our business. These additional healthcare regulations could affect our current and future arrangements with healthcare professionals, principal investigators, consultants, customers and third party payors. Such laws include, without limitation, state and federal anti kickback, fraud and abuse, false claims, privacy and security, and physician payment sunshine laws.

The federal Anti Kickback Statute makes it illegal for any person or entity, including a prescription drug manufacturer (or a party acting on its behalf) to knowingly and willfully, directly or indirectly, solicit, receive, offer, or pay any remuneration that is intended to induce the referral of business, including the purchase, order, or lease of any good, facility, item or service for which payment may be made under a federal healthcare program, such as Medicare or Medicaid. The term “remuneration” has been broadly interpreted to include anything of value. Several courts have interpreted the statute’s intent requirement to mean that if any one purpose of an arrangement involving remuneration is to induce referrals of federal healthcare covered business, the Anti Kickback Statute has been violated. The Patient Protection and Affordable Care Act of 2010, as amended by the Health Care and Education Reconciliation Act, collectively the Affordable Care Act or ACA, among other things, amended the intent requirement of the federal Anti Kickback Statute such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate, in order to commit a violation.

Federal civil and criminal false claims and false statement laws, including the federal civil False Claims Act and its whistleblower or qui tam provisions that permit private individuals to bring an action on behalf of the government to enforce the civil False Claims Act, prohibit, among other things, any person or entity from knowingly presenting, or causing to be presented, for payment to, or approval by, federal programs, including Medicare and Medicaid, claims for items or services, including drugs, that are false or fraudulent or not provided as claimed. Entities can be held liable under these laws if they are deemed to “cause” the submission of false or fraudulent claims by, for example, providing inaccurate billing or coding information to customers, promoting a product off label, or for providing medically unnecessary services or items. In addition, a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal civil False Claims Act. Criminal prosecution is also possible for making or presenting a false, fictitious or fraudulent claim to the federal government.

The federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, created criminal and civil liability for, among other things, knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program, including private third party payors, knowingly and willfully embezzling or stealing from a healthcare benefit program, willfully obstructing a criminal investigation of a healthcare offense, and knowingly and willfully falsifying, concealing or covering up a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services.

HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and their implementing regulations, imposes obligations, including mandatory contractual terms, with respect to safeguarding the privacy, security, transmission and breach reporting of individually identifiable health information, upon entities subject to the law, such as health plans, healthcare clearinghouses and certain healthcare providers and their respective business associates that perform services for them that involve individually identifiable health information. HITECH also created new tiers of civil monetary penalties, amended HIPAA to make civil and criminal

penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in U.S. federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions.

The federal Physician Payments Sunshine Act requires certain manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program, with specific exceptions, to report annually to the Centers for Medicare & Medicaid Services, or CMS,

information related to payments or other transfers of value made to physicians and teaching hospitals, and applicable manufacturers and applicable group purchasing organizations to report annually to CMS ownership and investment interests held by physicians and their immediate family members.

Analogous state and foreign laws and regulations, such as state anti kickback and false claims laws, may apply to sales or marketing arrangements and claims involving healthcare items or services reimbursed by non governmental third-party payors, including private insurers. Additionally, we may be subject to state laws that require pharmaceutical companies to comply with the pharmaceutical industry's voluntary compliance guidelines and the relevant compliance guidance promulgated by the federal government. Further, we may be subject to state laws that require drug manufacturers to report information related to payments and other transfers of value to physicians, other healthcare providers and healthcare entities, or marketing expenditures, as well as state and local laws that require the registration of pharmaceutical sales representatives; state laws that require the reporting of information related to drug pricing; and state and foreign laws governing the privacy and security of health information, including the General Data Protection Regulation, or GDPR, from the European Union, or EU, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Efforts to ensure that our current and future business arrangements will comply with applicable healthcare, privacy and data security laws and regulations will involve substantial costs. For example, the GDPR, which became effective on May 25, 2018, imposes several requirements relating to the consent of the individuals to whom the personal data relates, the information provided to the individuals, the security and confidentiality of the personal data, data breach notification and the use of third-party processors in connection with the processing of personal data. The GDPR also imposes strict rules on the transfer of personal data out of the EU, provides an enforcement authority and authorizes the imposition of large penalties for noncompliance, including the potential for fines of up to €20 million or 4% of the annual global revenues of the noncompliant company, whichever is greater. The GDPR has increased our responsibility and potential liability in relation to personal data that we process or control compared to prior EU law, including in clinical trials, and we may be required to put in place additional mechanisms to ensure compliance with the GDPR, which could divert management's attention and increase our cost of doing business. Likewise, we expect that there will continue to be new proposed laws, regulations and industry standards relating to privacy and data protection in the United States, the EU and other jurisdictions, such as the California Consumer Privacy Act of 2018, which has been characterized as the first "GDPR-like" privacy statute to be enacted in the United States because it mirrors a number of the key provisions in the GDPR, that will go into effect beginning January 1, 2020, and we cannot determine the impact that such laws, regulations and standards will have on our business. In any event, it is possible that governmental authorities will conclude that our business practices do not comply with current or future statutes, regulations, agency guidance or case law involving applicable healthcare or privacy laws, including the GDPR, in light of the lack of applicable precedent and regulations. Federal and state enforcement bodies have recently increased their scrutiny of interactions between healthcare companies and healthcare providers, which has led to a number of investigations, prosecutions, convictions and settlements in the healthcare industry.

If our operations are found to be in violation of any of these or any other healthcare and privacy-related regulatory laws that may apply to us, we may be subject to significant penalties, including the imposition of significant civil, criminal and administrative penalties, damages, monetary fines, disgorgement, imprisonment, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, reputational harm, diminished profits and future earnings, additional reporting requirements and oversight if we become subject to a corporate integrity agreement or similar agreement to resolve allegations of non-compliance with these laws, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations. Defending against any such actions can be costly, time-consuming and may require significant financial and personnel resources. Therefore, even if we are successful in defending against any such actions that may be brought against us, our business may be impaired.

Reimbursement and Healthcare Reform

Significant uncertainty exists as to the coverage and reimbursement status of any product candidate that receives regulatory approval. In the United States and markets in other countries, sales of imetelstat, if approved for commercial sale, will depend, in part, on the extent to which third party payors provide coverage and establish adequate reimbursement levels for imetelstat.

In the United States, third party payors include federal and state healthcare programs, government authorities, private managed care providers, private health insurers and other organizations. There has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and federal and state legislative activity designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, reduce the cost of drugs under Medicare, and reform government program reimbursement methodologies for drugs, some of which are included in the Trump Administration's budget proposal for fiscal year 2019. Additionally, at the federal level, the Trump Administration released a "Blueprint" that contains additional proposals to increase manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products and reduce the out of pocket costs of drug products paid by consumers. On January 31, 2019, the U.S. Department of Health and Human Services, Office of Inspector General, proposed modifications to federal Anti-Kickback Statute safe harbors which, among other things, will affect rebates paid by manufacturers to Medicare Part D plans, the purpose of which is to further reduce the cost of drug products to consumers. While a number of these and other proposed measures may require authorization through additional legislation to become effective, Congress and the Trump Administration have each indicated that they will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, to encourage importation from other countries and bulk purchasing. Further, third party payors are increasingly challenging the price, examining the medical necessity and reviewing the cost effectiveness of medical drug products and medical services, in addition to questioning their safety and efficacy. Such payors may limit coverage to specific drug products on an approved list, also known as a formulary, which might not include all of the FDA approved drugs for a particular indication. We may need to conduct expensive pharmacoeconomic studies in order to demonstrate the medical necessity and cost effectiveness of imetelstat, in addition to the costs required to obtain the FDA approvals. Nonetheless, imetelstat may not be considered medically necessary or cost effective.

Moreover, the process for determining whether a third party payor will provide coverage for a drug product may be separate from the process for setting the price of a drug product or for establishing the reimbursement rate that such a payor will pay for the drug product. A payor's decision to provide coverage for a drug product does not imply that an adequate reimbursement rate will be approved. Further, one payor's determination to provide coverage for a drug product does not assure that other payors will also provide coverage for the drug product, as there is no uniform coverage and reimbursement policy among third-party payors in the United States. Adequate third party reimbursement may not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in imetelstat.

The United States and some foreign jurisdictions are considering or have enacted legislative and regulatory proposals to contain healthcare costs, as well as to improve quality and expand access. For example, in March 2010, the ACA, was signed into law that included a number of provisions of importance to the biopharmaceutical industry. Since its enactment, there have been judicial and Congressional challenges to certain aspects of the ACA, as well as recent efforts by the Trump Administration to repeal or replace certain aspects of the ACA. For example, since January 2017, President Trump has signed two executive orders and other directives designed to delay, circumvent, or loosen certain requirements mandated by the ACA. Concurrently, Congress has considered legislation that would repeal or repeal and replace all or part of the ACA. While Congress has not passed comprehensive repeal legislation, two bills affecting the implementation of certain taxes under the ACA have been signed into law. The Tax Cuts and Jobs Act of 2017, or Tax Act, includes a provision which repealed, effective January 1, 2019, the tax-based shared responsibility payment imposed by the ACA on certain individuals who fail to maintain qualifying health coverage for all or part of a year, that is commonly referred to as the "individual mandate." On January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain

ACA-mandated fees.

The Bipartisan Budget Act of 2018, among other things, amended the ACA, effective January 1, 2019, to close the coverage gap in most Medicare Part D drug plans, commonly referred to as the “donut hole”. In July 2018, CMS published a final rule permitting further collections and payments to and from certain ACA qualified health plans and health insurance issuers under the ACA risk adjustment program in response to the outcome of federal district court litigation regarding the method CMS uses to determine this risk adjustment. On December 14, 2018, a Texas U.S.

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District Court Judge ruled that the ACA is unconstitutional in its entirety because the “individual mandate” was repealed by Congress as part of the Tax Act. While the Texas U.S. District Court Judge, as well as the Trump administration and CMS, have stated that the ruling will have no immediate effect pending appeal of the decision, it is unclear how this decision, subsequent appeals, and other efforts to repeal and replace the ACA will impact the ACA. We expect that other healthcare reform measures that may be adopted in the future may result in more rigorous coverage criteria and lower reimbursement, and additional downward pressure on the price that may be charged for imetelstat.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. For example, in August 2011, the Budget Control Act of 2011 was enacted, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend to Congress proposals in spending reductions. The Joint Select Committee on Deficit Reduction did not achieve a targeted deficit reduction of at least \$1.2 trillion for fiscal years 2012 through 2021, triggering the legislation’s automatic reduction to several government programs. This includes aggregate reductions to Medicare payments to providers of up to 2% per fiscal year, which went into effect beginning on April 1, 2013 and, due to subsequent legislative amendments to the statute, including the Bipartisan Budget Act of 2018, will stay in effect through 2027 unless additional Congressional action is taken. Additionally, in January 2013, the American Taxpayer Relief Act of 2012 was signed into law, which, among other things, reduced Medicare payments to several providers, including hospitals and imaging centers. More recently, there has been heightened governmental scrutiny in the United States to control the rising cost of healthcare.

Executive Officers of the Company

The following table sets forth certain information with respect to our executive officers as of January 31, 2019:

Name	Age	Position
John A. Scarlett, M.D.	67	President, Chief Executive Officer and Chairman of the Board
Olivia K. Bloom	50	Executive Vice President, Finance, Chief Financial Officer and Treasurer
Melissa A. Kelly Behrs	55	Executive Vice President and Chief Business Officer
Andrew J. Grethlein, Ph.D.	54	Executive Vice President and Chief Operating Officer
Aleksandra Rizo, M.D., Ph.D.	44	Executive Vice President and Chief Medical Officer
Stephen N. Rosenfield, J.D.	69	Executive Vice President, Chief Legal Officer and Corporate Secretary

John A. Scarlett, M.D., has served as our Chief Executive Officer and a director since September 2011 and President since January 2012 and was appointed to Chairman of the Board in December 2018. Dr. Scarlett has served as a director for Chiasma, Inc., a biopharmaceutical company focused on transforming injectable drugs into oral medications, since February 2015 and CytomX Therapeutics, Inc., a biopharmaceutical company focused on developing antibody therapeutics for the treatment of cancer, since June 2016. Prior to joining Geron, Dr. Scarlett served as President, Chief Executive Officer and a member of the board of directors of Proteolix, Inc., a privately held, oncology oriented biopharmaceutical company, from February 2009 until its acquisition by Onyx Pharmaceuticals, Inc., an oncology oriented biopharmaceutical company, in November 2009. From February 2002 until its acquisition by Ipsen, S.A. in October 2008, Dr. Scarlett served as the Chief Executive Officer and a member of the board of directors of Tercica, Inc., an endocrinology oriented biopharmaceutical company, and also as its President from February 2002 through February 2007. From March 1993 to May 2001, Dr. Scarlett served as President and Chief Executive Officer of Sensus Drug Development Corporation. In 1995, he co founded Covance Biotechnology Services, Inc., a contract biopharmaceutical manufacturing operation, and served as a member of its

board of directors from inception to 2000. From 1991 to 1993, Dr. Scarlett headed the North American Clinical Development Center and served as Senior Vice President of Medical and Scientific Affairs at Novo Nordisk Pharmaceuticals, Inc., a wholly owned subsidiary of Novo Nordisk A/S. Dr. Scarlett received his B.A. degree in chemistry from Earlham College and his M.D. from the University of Chicago, Pritzker School of Medicine.

Olivia K. Bloom has served as our Executive Vice President, Finance since February 2014, Chief Financial Officer since December 2012 and Treasurer since February 2011. Ms. Bloom previously served as our Senior Vice President, Finance from December 2012 to February 2014, Chief Accounting Officer from September 2010 to December 2012 and Vice President, Finance from January 2007 to December 2012. Ms. Bloom joined the Company in 1994 as a Senior Financial Analyst and from 1996 to 2011 served as our Contoller. Prior to joining Geron, Ms. Bloom started her career in public accounting at KPMG Peat Marwick and became a Certified Public Accountant in 1994. Ms. Bloom graduated Phi Beta Kappa with a B.S. in Business Administration from the University of California at Berkeley.

Melissa A. Kelly Behrs has served as our Executive Vice President and Chief Business Officer since January 2019. Previously, she was our Executive Vice President, Business Development and Portfolio & Alliance Management, from February 2014 to January 2019, and our Senior Vice President, Portfolio and Alliance Management from September 2012 to February 2014. Ms. Behrs joined Geron in November 1998 as Director of Corporate Development. Since then, she has also served in various managerial positions, including General Manager, R&D Technologies; Vice President, Corporate Development; Senior Vice President, Therapeutic Development, Oncology; and Senior Vice President, Strategic Portfolio Management. From 1990 to 1998, Ms. Behrs worked at Genetics Institute, Inc., a biotechnology research and development company, serving initially as Assistant Treasurer and then as Associate Director of Preclinical Operations where she was responsible for all business development, regulatory, and project management activities for the Preclinical Development function. Ms. Behrs received a B.S. from Boston College and an M.B.A. from Babson College.

Andrew J. Grethlein, Ph.D., has served as our Executive Vice President and Chief Operating Officer since January 2019. Previously, he served as our Executive Vice President, Development and Technical Operations, from July 2014 to January 2019. He joined Geron in September 2012 as our Executive Vice President, Technical Operations. Prior to joining Geron, Dr. Grethlein was Executive Vice President and Chief Operating Officer for Inspiration Biopharmaceuticals, a biopharmaceutical company, from January 2010 to September 2012. From October 2008 until January 2010, Dr. Grethlein was Senior Vice President of Biotechnology and Portfolio Management Team Leader for Hematology at Ipsen S.A., a global specialty pharmaceutical company. His responsibilities at Ipsen included planning and execution of worldwide strategy for product and portfolio development in the hematologic therapeutic area. From 2003 to 2008, Dr. Grethlein served as Senior Vice President of Pharmaceutical Operations at Tercica, Inc., an endocrinology oriented biopharmaceutical company, where he was a member of the senior executive team that governed corporate strategy, business planning and company operations, and had responsibility for all manufacturing and quality functions. Before joining Tercica, Dr. Grethlein served in various positions at Elan Corporation, a biotechnology company, from 1997 to 2003, including as Senior Director, South San Francisco Pharmaceutical Operations. From 1995 to 1997, Dr. Grethlein served as Manager, Biologics Development and Manufacturing, for Athena Neurosciences, Inc., a pharmaceutical company. Prior to this, he served in various engineering positions for the Michigan Biotechnology Institute, a nonprofit technology research and business development corporation. Dr. Grethlein received his A.A. degree in liberal arts from Simon's Rock Early College, his B.S. in biology from Bates College, and his M.S. and Ph.D. in chemical engineering from Michigan State University.

Aleksandra Rizo, M.D., Ph.D., has served as our Executive Vice President and Chief Medical Officer since January 2019. Prior to joining Geron, Dr. Rizo was Executive Director, Strategy and Clinical Lead at Celgene Corporation, a biopharmaceutical company, from March 2018 to January 2019, where she led submission activities and participated in strategic and business development initiatives. From October 2008 to March 2018, Dr. Rizo served in a number of oncology drug development functions at Janssen Research and Development, LLC, a pharmaceutical company, including Senior Director, Compound Development Team Leader for all Phase 1 myeloid assets, and Global Clinical Leader for all late-stage myeloid assets, including imetelstat from November 2014 to March 2018, as well as Global Clinical Leader for the ibrutinib mantle cell lymphoma program. In these roles, she had oversight and leadership responsibilities for overall clinical development strategy, study designs, execution and data interpretation. In addition,

Dr. Rizo was a core member of Janssen's Hematology Strategy Team where she participated and led diligence projects in hematology. During her initial tenure with Janssen, Dr. Rizo also worked on a variety of Velcade clinical trials in lymphoma and multiple myeloma. Dr. Rizo holds an M.D. from the University Ss Cyril and Methodius, Skopje, Macedonia, where she also completed a residency in internal medicine/hematology. She also has a Ph.D. in human leukemic stem cell biology from the University of Groningen, Groningen, Netherlands, and a Ph.D. in mouse stem cell biology from the University of Tokyo, Tokyo, Japan.

Stephen N. Rosenfield, J.D., has served as our Executive Vice President, Chief Legal Officer and Corporate Secretary since January 2019. Previously, he served as our Executive Vice President, General Counsel and Corporate Secretary from February 2012 to January 2019, General Counsel and Secretary since January 2012 and Secretary since October 2011. From July 2009 to February 2012, Mr. Rosenfield served as a consultant to private companies. From June 2004 until June 2009, Mr. Rosenfield held several positions at Tercica, Inc., an endocrinology oriented biopharmaceutical company, and through its acquisition by Ipsen, S.A. in October 2008, including General Counsel and Secretary. Prior to joining Tercica, Mr. Rosenfield served as the Executive Vice President of Legal Affairs, General Counsel and Secretary of InterMune, Inc., a biotechnology company that focused on pulmonology and fibrotic diseases. Prior to joining InterMune, Mr. Rosenfield was an attorney at Cooley LLP, an international law firm, where he served as outside counsel for biotechnology and technology clients. Mr. Rosenfield received a B.S. from Hofstra University and a J.D. from Northeastern University School of Law.

Employees

As of December 31, 2018, we had 17 full time employees and one part time employee. One of our employees holds a Ph.D. degree and seven hold other advanced degrees. Of this current total workforce, three employees were engaged in, or directly supported, our research and development activities, and 15 employees were engaged in business development, legal, finance and administration. None of our employees are covered by a collective bargaining agreement; nor have we experienced work stoppages. We consider relations with our employees to be good.

Corporate Information

Geron Corporation was incorporated in the State of Delaware on November 28, 1990.

Available Information

Our internet address is www.geron.com. Information included on our website is not part of this annual report on Form 10 K. We make available free of charge on our website our annual reports on Form 10 K, quarterly reports on Form 10 Q, current reports on Form 8 K, and all amendments to those reports as soon as reasonably practicable after such material is electronically filed with or furnished to the United States Securities and Exchange Commission, or the SEC. In addition, copies of our annual reports are available free of charge upon written request.

ITEM 1A. RISK FACTORS

Our business is subject to various risks and uncertainties that may have a material adverse effect on our business, financial condition or results of operations. You should carefully consider the risks and uncertainties described below, together with all of the other information included in this annual report on Form 10-K. Our business faces significant risks and uncertainties, and those described below may not be the only risks and uncertainties we face. Additional risks and uncertainties not presently known to us or that we currently believe are immaterial may also significantly impair our business, financial condition or results of operations. If any of these risks or uncertainties occur, our business, financial condition or results of operations could suffer, the market price of our common stock could decline and you could lose all or part of your investment in our common stock.

RISKS RELATED TO THE DEVELOPMENT OF IMETELSTAT

Our future success depends solely on imetelstat, our only product candidate, and we cannot be certain that we will be able to continue to develop imetelstat or advance imetelstat to subsequent clinical trials, or that we will be able to receive regulatory approval for imetelstat on a timely basis, or at all.

Imetelstat is our sole product candidate, upon whose success we are wholly dependent. We do not have any other products or product candidates. Our ability to develop imetelstat to and through regulatory approval and commercial launch is subject to significant risks and uncertainties, including, among other things, our ability to:

- cause the IND for imetelstat to be maintained without such IND being placed on full or partial clinical hold by the FDA;
- generate additional safety and efficacy data from existing and potential future clinical trials of imetelstat, providing a positive benefit-risk profile that supports the continued and future development of imetelstat in hematologic myeloid malignancies;
- ascertain that the use of imetelstat does not result in significant systemic or organ toxicities, including hepatotoxicity, or other safety issues resulting in an unacceptable benefit-risk profile;
 - develop clinical plans for, and successfully enroll and complete, potential future clinical trials of imetelstat in hematologic myeloid malignancies, including the Phase 3 portion of IMerge;
- collaborate successfully with clinical trial sites, academic institutions, clinical research organizations, physician investigators and other third parties;
- obtain required regulatory clearances and approvals for imetelstat; for example, it is uncertain: whether the FDA and regulatory authorities in other countries will require us to obtain and submit additional non-clinical, manufacturing, or clinical data to proceed with any potential future clinical trials, how the FDA and other regulatory authorities will interpret safety and efficacy data from any clinical trial, including from IMbark or IMerge, what scope and type of clinical development and other data will be required before the FDA and other regulatory authorities might grant us marketing approval, if any, and what the length of time and cost for us will be to complete any such requirements;
- enter into and maintain arrangements with third parties to provide services needed to further research and develop imetelstat, including maintaining the agreement with our CRO, or to manufacture imetelstat, in each case at commercially reasonable costs;
- enter into and maintain arrangements with third parties, or establish internal capabilities, to provide sales, marketing and distribution functions in compliance with applicable laws;
- obtain appropriate coverage and reimbursement levels for the cost of imetelstat from governmental authorities, private health insurers and other third-party payors;
- maintain and enforce adequate intellectual property protection for imetelstat;

maintain adequate financial resources and personnel to advance imetelstat to and through potential future clinical trials, regulatory approval and commercial launch; and

- obtain funding necessary to fund our operations and to advance the development of imetelstat on commercially reasonable terms, including completion of the Phase 3 portion of IMerge and potential clinical development of other indications.

If we are not able to successfully achieve the above-stated goals and overcome other challenges that we may encounter in the research, development, manufacturing and potential commercialization of imetelstat, we may be forced to abandon our development of imetelstat, which would severely harm our business and prospects, and might cause us to cease operations.

Commencement of potential future clinical trials of imetelstat, including the Phase 3 portion of IMerge, and completion of the extension phase of IMbark and the Phase 2 portion of IMerge, could be interrupted, further delayed or abandoned for a variety of reasons.

Currently, there are two active clinical trials of imetelstat, the extension phase of IMbark and the Phase 2 portion of IMerge. Completion of these clinical trials, and the commencement of any potential future clinical trials of imetelstat, including the Phase 3 portion of IMerge, could be interrupted, delayed or abandoned for a variety of reasons, including as a result of failures or delays in:

- the comprehensive transition of the imetelstat program from Janssen to us, as discussed in more detail under the heading, “Risks Related to Transition of the Imetelstat Program from Janssen to Geron”;
- demonstrating sufficient safety and efficacy of imetelstat in IMerge and any potential future clinical trials, without safety issues, side effects or dose-limiting toxicities, including any additional or more severe safety issues in addition to those that have been observed to date in previous or ongoing clinical trials related to imetelstat, whether or not in the same indications or therapeutic areas;
- obtaining or maintaining regulatory clearances in the United States or other countries to conduct clinical trials, such as obtaining or maintaining regulatory clearances to commence, conduct or modify current or potential future clinical trials of imetelstat, in a timely manner, or at all, which could, for example, cause the anticipated commencement of the Phase 3 portion of IMerge to be delayed beyond mid-year 2019 or prevent us from commencing or completing the Phase 3 portion of IMerge;
- maintaining the IND for imetelstat without such IND being placed on full or partial clinical hold, suspended or subject to other requirements by the FDA or other regulatory authorities;
- properly (i) completing the extension phase of IMbark, including collecting data about serious adverse events and overall survival from the extension phase of IMbark; (ii) completing the Phase 2 portion of IMerge, including assessing the durability of RBC-TI responses; and (iii) designing, enrolling, conducting and completing the Phase 3 portion of IMerge, and promptly or adequately reporting data from such trials;
- determining, after consultations with experts in MF and discussions with regulatory authorities, whether the results from the IMbark primary analysis provide a feasible registration path, if any, for imetelstat in Intermediate-2 or High risk MF patients who have relapsed after or are refractory to prior treatment with a JAK inhibitor;
- obtaining or accessing necessary clinical data in accordance with appropriate clinical or quality practices to ensure complete data sets;
- responding to safety findings by the data review committees of current clinical trials, including the extension phase of IMbark and the Phase 2 portion of IMerge, and safety or futility findings by the data review committees of potential future clinical trials of imetelstat, such as the Phase 3 portion of IMerge, based on emerging data occurring during such clinical trials, such as significant systemic or organ toxicities, including severe cytopenias, hepatotoxicity, fatal bleeding with or without any associated thrombocytopenia, patient injury or death, or other safety issues, resulting in an unacceptable benefit-risk profile;
- obtaining funding on commercially reasonable terms necessary to advance the development of imetelstat;

- manufacturing sufficient quantities of imetelstat or other clinical trial materials in a manner that meets the quality standards of the FDA and other regulatory authorities, and responding to any disruptions to drug supply, clinical trial materials or quality issues that may arise;

- ensuring the ability to manufacture imetelstat at acceptable costs for potential Phase 3 clinical trials and commercialization;

- obtaining sufficient quantities of any study-related treatments, materials (including comparator products, placebo or combination therapies) or ancillary supplies;

- obtaining acceptance by regulatory authorities of manufacturing changes, as well as successfully implementing any such manufacturing changes;

- complying with current and future regulatory requirements, policies or guidelines, including domestic and international laws and regulations pertaining to fraud and abuse, transparency, and the privacy and security of health information;

- reaching agreement on acceptable terms and on a timely basis, if at all, with collaborators and vendors located in the United States or foreign jurisdictions, including contract research organizations, laboratory service providers and clinical trial sites, on all aspects of clinical development;

- obtaining timely review and clearances by regulatory authorities of future protocol amendments which may be sought for the Phase 3 portion of IMerge and potential future clinical trials of imetelstat, including responding to questions or comments from these authorities in a timely and adequate manner, which could, for example, cause the anticipated commencement of the Phase 3 portion of IMerge to be delayed beyond mid-year 2019 or prevent us from commencing or completing the Phase 3 portion of IMerge; and

- obtaining institutional review board or ethics committee approval of clinical trial protocols or protocol amendments, including any future refinements to the trial design we may seek for the Phase 3 portion of IMerge, which could, for example, cause the anticipated commencement of the Phase 3 portion of IMerge to be delayed beyond mid-year 2019 or prevent us from commencing or completing the Phase 3 portion of IMerge.

Failures or delays with respect to any of these events could adversely affect our ability to continue or successfully complete the extension phase of IMbark or the Phase 2 portion of IMerge or to commence potential future clinical trials of imetelstat, including the Phase 3 portion of IMerge, which could increase development costs, or interrupt, further delay or halt our development or commercialization of imetelstat, any of which could severely and adversely affect our financial results, business and business prospects, and the future of imetelstat, and might cause us to cease operations.

Imetelstat may cause, or have attributed to it, undesirable or unintended side effects or other adverse events that further delay or prevent the commencement and/or completion of clinical trials for imetelstat, further delay or prevent its regulatory approval, or limit its commercial potential.

Imetelstat may cause, or have attributed to it, undesirable or unintended side effects or other adverse events affecting its safety or efficacy that could interrupt, further delay or halt current or potential future clinical trials of imetelstat. For example, adverse events and dose-limiting toxicities observed in previous clinical trials of imetelstat include:

- hematologic toxicities, such as profound and/or prolonged thrombocytopenia or neutropenia, including one case of febrile neutropenia after prolonged myelosuppression with intracranial hemorrhage resulting in patient death, which the investigator assessed as possibly related to imetelstat;

- bleeding events, with or without thrombocytopenia;

- liver function test, or LFT, abnormalities, the clinical significance and long-term consequences of which are currently undetermined;

- gastrointestinal events;

- infections;

- muscular and joint pain;
- fatigue; and
- infusion reactions.

Such adverse events and other safety issues, including deaths, were also observed in IMbark and the Phase 2 portion of IMerge. If patients in any potential future clinical trials of imetelstat, including the Phase 3 portion of IMerge, experience similar or more severe adverse events, or new or unusual adverse events, or if the FDA or other regulatory authorities determine that efficacy and safety data in current or potential future clinical trials of imetelstat do not support an adequate benefit-risk profile to justify continued treatment of patients, then the FDA or other regulatory authorities may again place the IND for imetelstat on clinical hold, as occurred in March 2014.

Further, clinical trials by their nature examine the effect of a potential therapy in a sample of the potential future patient population. As such, clinical trials conducted with imetelstat, to date and in the future, may not uncover all possible adverse events that patients treated with imetelstat may experience. Because remaining patients in the treatment phase continue to receive imetelstat, in the extension phase of IMbark and in the Phase 2 portion of IMerge, additional or more severe toxicities or safety issues, including additional serious adverse events and dose-limiting toxicities, may be observed as patient treatment continues and more data become available. In addition, since additional data are being generated from the extension phase of IMbark and Part 1 of IMerge, the benefit-risk profile of imetelstat will continue to be assessed, including the risk of hepatotoxicity, severe cytopenias, fatal bleeding with or without any associated thrombocytopenia, patient injury or death, and any other severe adverse effects that may be associated with life-threatening clinical outcomes. If such toxicities or other safety issues in any clinical trial of imetelstat are determined by us, the FDA or any other regulatory authority to result in an unacceptable benefit-risk profile, then:

- additional information supporting the benefit-risk profile of imetelstat may be requested by the FDA or other regulatory authorities and if any such information supplied by Janssen, or by us following the transition of the imetelstat program to us is not deemed acceptable, current clinical trials of imetelstat could be suspended, terminated, or placed on clinical hold by the FDA or other regulatory authorities;
- the ability to retain enrolled patients in current clinical trials may be negatively affected, resulting in incomplete data sets and the inability to adequately assess the benefit-risk profile of imetelstat in a specific patient population; or
- additional, unexpected clinical trials or non-clinical studies may be required to be conducted.

The occurrence of any of these events could interrupt, further delay, or halt, any development and commercialization of imetelstat by us, which would have a severe adverse effect on our results of operations, financial condition, business prospects and the future of imetelstat, any of which might cause us to cease operations.

Results obtained in prior non-clinical studies and clinical trials do not predict success in later clinical trials. Likewise, preliminary data from clinical trials should be considered carefully and with caution since final data may be materially different from preliminary data, particularly as more patient data become available.

Success in non-clinical testing and early clinical trials does not ensure that later clinical trials will be successful. We cannot be certain that any of the prior, current or potential future clinical trials of imetelstat will generate sufficient, consistent or adequate efficacy and safety data demonstrating a positive benefit-risk profile, which would be necessary to obtain regulatory approval to market imetelstat in any indication. Product candidates in later stages of clinical trials may fail to show the desired benefit-risk profile despite having progressed through non-clinical studies and initial clinical trials. Other companies in the biopharmaceutical industry have frequently suffered significant setbacks in later clinical trials, even after achieving promising results in earlier non-clinical studies or clinical trials.

Safety and efficacy data from previous or current imetelstat clinical trials in hematologic myeloid malignancies should not be relied upon as predictive or indicative of future clinical trial results. For example, complete and partial remissions observed in the Pilot Study suggested potential disease-modifying activity of imetelstat in the MF patient population enrolled in the Pilot Study. However, similar activity was not observed in the MF patients enrolled in IMbark, as shown by the one partial remission observed in the IMbark primary analysis. We believe that differences in the IMbark study design when compared to the Pilot Study design, such as more restrictive patient enrollment criteria requiring either documented objective lack of response to a JAK inhibitor or evidence of progressive disease while on treatment with a JAK inhibitor, may have contributed to the data observed in IMbark differing significantly from data reported from the Pilot Study, but we cannot assure you that any future clinical trials of imetelstat in MF will yield results comparable to IMbark or the Pilot Study. In addition, the potential improvement in survival observed in the 9.4 mg/kg dosing arm in IMbark will need to be further assessed in a Phase 3 clinical trial comparing imetelstat to a control therapy, and similar results, including potential improvement in survival, if any, with respect to any patient population or patient population subgroup, may not be observed.

Similarly, in the Phase 2 portion of IMerge, the initial data review for the expansion cohort conducted by Janssen in the second quarter of 2018, which Janssen called a “data snapshot,” exhibited 8-week RBC-TI rate of 28%, while the 13-patient initial cohort exhibited 8-week RBC-TI rate of 54% resulting in an overall 8-week RBC-TI rate of 37% for the combined cohorts. We believe the observed difference in 8-week RBC-TI rate between the 13-patient initial cohort and the 25-patient expansion cohort may be attributable to factors such as the maturity of the data at the time of the data snapshot since the median follow-up time of the expansion cohort at the time of the data snapshot was less than half the length of time the 13-patient initial cohort had been followed when their data were first reported, or the higher overall baseline transfusion burden of the expansion cohort, but we cannot assure you that the combined 8-week RBC-TI rate observed in the Phase 2 portion of IMerge will improve with longer follow-up, or at all, or that the 8-week RBC-TI rate of patients enrolled in the Phase 3 portion of IMerge, if any, will be comparable to what has been observed in the 13-patient initial cohort, the expansion cohort, or the combined cohorts.

Additional or updated safety and efficacy data from current or potential future imetelstat clinical trials may result in a benefit-risk profile that does not justify continued development of imetelstat in a particular patient population, or at all. For example, because patients remaining in the treatment phase continue to receive imetelstat, in the extension phase of IMbark and the Phase 2 portion of IMerge, efficacy and safety data continue to be generated. Such additional data could result in a lower benefit-risk profile than initially expected, which could hinder the commencement, completion and potential success in the Phase 3 portion of IMerge, or could cause us to abandon further development of imetelstat entirely. Data from the Phase 3 portion, of IMerge could materially differ from the overall conclusions reported for the Phase 2 portion of IMerge. In general, Phase 3 clinical trials with larger numbers of patients or longer durations of therapy may fail to replicate efficacy results observed in earlier clinical trials, or may reveal safety concerns that were not identified in smaller or shorter trials, any of which could adversely affect future development prospects of imetelstat.

From time-to-time, safety and efficacy data from previous and current imetelstat clinical trials have been reported or announced by us, clinical investigators or Janssen. For example, preliminary data from the Phase 2 portion of IMerge was presented at the ASH annual meetings in December 2017 and December 2018, and at the EHA annual congress in June 2018. We expect similar reports or announcements of safety and efficacy data from us or clinical investigators as data matures in current imetelstat clinical trials and from potential future clinical trials. Preliminary or interim results may not be reproduced in any potential future clinical trials of imetelstat, and thus should be considered carefully and with caution, and not relied upon as indicative of future clinical results. Material adverse differences in final data, compared to preliminary or interim data, could severely and adversely affect our financial results, business and business prospects, and the future of imetelstat, and might cause us to cease operations.

The research and development of imetelstat is subject to numerous risks and uncertainties.

The science and technology of telomere biology, telomerase and our proprietary oligonucleotide chemistry are relatively new. There is no precedent for the successful commercialization of a therapeutic product candidate based on these technologies. Significant research and development activities will be necessary to further develop imetelstat, which is our sole product candidate, which may take years to accomplish, if at all.

Because of the significant scientific, regulatory and commercial challenges that must be overcome to successfully research, develop and commercialize imetelstat, the development of imetelstat in hematologic myeloid malignancies, including MF and MDS, or any other indications, may be further delayed or abandoned, even after significant resources have been expended on it. Examples of such situations include:

- the discontinuation of our Phase 2 clinical trial of imetelstat in metastatic breast cancer in September 2012;
- the discontinuation of our development of imetelstat in solid tumors with short telomeres in April 2013;
- Janssen's decisions in the third quarter of 2016 to close the 4.7 mg/kg dosing arm in IMbark to new patient enrollment and to suspend enrollment in the 9.4 mg/kg dosing arm in IMbark because an insufficient number of patients in the 9.4 mg/kg dosing arm met the protocol defined interim efficacy criteria at 12 weeks;
- Janssen's decision in the third quarter of 2017 to expand the Phase 2 portion of IMerge to enroll additional lower risk MDS patients in a target patient population; and
- Janssen's decision in September 2018 to terminate the Collaboration Agreement.

Further delay, suspension or abandonment of the development of imetelstat in hematologic myeloid malignancies, including further delays resulting from the termination of the Collaboration Agreement, transition of the imetelstat program from Janssen to us, and our ability to successfully plan for and commence future clinical trials of imetelstat, including the Phase 3 portion of IMerge, could have a material adverse effect on the future of imetelstat and our business prospects, and we might cease operations.

If we encounter difficulties enrolling or retaining patients in current or potential future clinical trials of imetelstat, including in the Phase 3 portion of IMerge, clinical development and commercialization activities could be further delayed or otherwise adversely affected, which would cause our business and business prospects to be severely harmed, and we might cease operations.

The timely completion of a clinical trial in accordance with its protocol depends, among other things, on the ability to enroll a sufficient number of patients who remain in the trial until its conclusion. For example, if we experience difficulties in retaining patients in the extension phase of IMbark, our ability to continue to assess OS would be adversely affected. If we experience difficulties in retaining patients in the Phase 2 portion of IMerge, our ability to continue to assess the durability of RBC-TI responses would be adversely affected. In addition, we may encounter challenges in enrolling and retaining patients in potential future clinical trials of imetelstat, including the Phase 3 portion of IMerge, for a variety of reasons. The enrollment and retention of patients depends on many factors, including:

- the patient eligibility criteria specified in the protocol;
- the size of the patient population required for analysis of the trial's primary endpoint;
- the proximity of patients to trial sites;
- the design of the trial;
- our ability to recruit and retain clinical trial investigators with the appropriate competencies and experience;
- clinicians' and patients' perceptions of the potential advantages of imetelstat, both in relation to other available therapies, including any new drugs that may be approved for the indications being investigated, and as a result of data reported from previous or current clinical trials of imetelstat;
- the ability to obtain and maintain patient consents; and
- the risk that patients enrolled in any imetelstat clinical trial will drop out of the trial before completion due to lack of efficacy, adverse side effects, investigator decision, slow progress to later stage clinical trials or personal issues.

In addition, potential future clinical trials of imetelstat, including the Phase 3 portion of IMerge, will compete, with other clinical trials for product candidates that are in the same therapeutic areas with imetelstat and such trials may also be conducted at the same clinical sites, and this competition will reduce the number and type of patients available to enroll or remain in the imetelstat clinical trials. Moreover, because imetelstat represents a departure from more commonly used methods for cancer treatment, potential patients and their doctors may be inclined to use conventional therapies, rather than enroll patients into imetelstat clinical trials, or may decide not to enroll, or may not recommend enrollment, in future clinical trials of imetelstat, based on efficacy and safety results reported to date and that may be reported in the future.

Delays in patient enrollment or the inability to retain or treat patients could result in increased costs, lead to incomplete data sets, or adversely affect the timing or outcome of current or potential future clinical trials of imetelstat, including the Phase 3 portion of IMerge, which could prevent completion of these trials and adversely affect the clinical development and potential commercialization of imetelstat. Such occurrences would severely and adversely affect our financial results, business and business prospects, and the future of imetelstat, and might cause us to cease operations.

We do not have experience as a company in conducting large-scale, late-stage clinical trials, such as the Phase 3 portion of IMerge or potential future similar trials, or in those functional areas that would be required for the successful commercialization of our sole product candidate, imetelstat.

We have no experience in conducting large-scale, late-stage clinical trials, such as the Phase 3 portion of IMerge, nor do we have experience with activities that would be required for the commercialization of imetelstat, should we receive future regulatory approval to do so. We cannot be certain that we will be able to design, enroll, conduct or complete the Phase 3 portion of IMerge, or any other future large-scale, late-stage clinical trial of imetelstat, in a timely fashion, or at all. Large-scale, late-stage clinical trials require additional financial resources and certain internal development experience that we do not currently possess, as well as increased reliance on third-party clinical investigators, CROs, service providers, vendors, suppliers and consultants. Relying on these third parties and establishing effective and collaborative relationships with them to conduct large-scale, late-stage clinical trials may cause further delays that are outside of our control. Any such further delays could have a material adverse effect on our business.

We also do not have commercialization capabilities. Developing an internal sales, marketing and distribution capability would be an expensive and time-consuming process, and will require additional management expertise. We may not be able to negotiate and enter into third-party marketing and distribution agreements on terms that are economically attractive, or at all. Even if we do enter into such agreements, third party marketers and distributors may not successfully market or distribute our sole product candidate, imetelstat.

Our inability to successfully conduct large-scale, late-stage clinical trials, such as the Phase 3 portion of IMerge or future similar trials, or to successfully establish commercialization capabilities for imetelstat if we receive future regulatory approval to do so, would severely and adversely affect our financial results, business and business prospects, and the future of imetelstat, and might cause us to cease operations.

We will rely on third parties to conduct our current and potential future clinical trials of imetelstat. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may be unable to continue the development of, obtain regulatory approval for, or commercialize imetelstat.

We do not have the ability to independently conduct clinical trials. Therefore, we will rely on medical institutions, clinical investigators, contract laboratories and other third parties, such as CROs, service providers, vendors, suppliers and consultants, to conduct clinical trials of imetelstat. The third parties with whom we contract for execution of our clinical trials will play a critical role in the conduct of these trials and the subsequent collection and analysis of data. However, these third parties are not our employees, and except for contractual duties and obligations, we have limited ability to control their performance, or the amount or timing of resources that they devote to imetelstat. For example, the CRO we have retained to support our clinical development activities will be critical to our development of imetelstat, including the Phase 3 portion of IMerge, and any failure by our CRO to perform its contractual obligations, or disputes with our CRO about the quality of its performance or other matters, could cause the anticipated commencement of the Phase 3 portion of IMerge to be delayed beyond mid-year 2019 or

prevent us from commencing or completing the Phase 3 portion of IMerge, or could otherwise further delay or halt our imetelstat development activities. These third parties may also have relationships with other commercial entities, some of which may compete with us. Under certain circumstances, these third parties may terminate their agreements with us without cause and upon immediate written notice.

Although we will rely on third parties to conduct any imetelstat clinical trials, including the Phase 3 portion of IMerge, we remain responsible for ensuring that each clinical trial is conducted in accordance with its investigational plan and protocol. Moreover, the FDA and foreign regulatory authorities require us to comply with regulations and standards, including regulations commonly referred to as good clinical practices, or GCPs, for conducting, monitoring, recording and reporting the results of clinical trials to ensure that the data and results are scientifically credible and accurate, and that patients are adequately informed of the potential risks of participating in clinical trials. Our ability to comply with these regulations and standards is contingent upon activities conducted by third parties, and if they fail to perform in accordance with contractual obligations and legal requirements, our development of imetelstat may be interrupted, further delayed or halted, which would have a severe adverse effect on our results of operations, financial condition, business prospects and the future of imetelstat, any of which might cause us to cease operations.

In addition, the execution of clinical trials and the subsequent compilation and analysis of the data produced, requires coordination among various parties. In order for these functions to be carried out effectively and efficiently, it is imperative that these parties communicate and coordinate with one another. If the quality or accuracy of the clinical data obtained, compiled or analyzed by third parties is compromised due to their failure to adhere to our clinical trial protocols or GCPs, or for any other reason, we may need to enter into new arrangements with alternative third parties, which could be difficult, costly or impossible. If third parties conducting our clinical trials do not perform their contractual duties or obligations, experience work stoppages, do not meet expected deadlines, terminate their agreements with us or need to be replaced, our clinical trials may be extended, delayed or terminated, or may be unsuccessful or need to be repeated, which could have a material adverse effect on our business and might cause us to cease operations.

RISKS RELATED TO TRANSITION OF THE IMETELSTAT PROGRAM FROM JANSSEN TO GERON

Encountering delays or difficulties in transitioning the imetelstat program from Janssen to us would prevent us from timely developing imetelstat, or preclude us from developing imetelstat at all, which could severely and adversely affect our financial results, business and business prospects, and might cause us to cease operations.

Under the terms of the Collaboration Agreement, Janssen is required to provide operational support for the imetelstat program during transition of the program to us. The transition process is expected to occur through September 2019, to enable the orderly transfer of all ongoing clinical, regulatory, medical affairs and non-clinical activities to us, including transfer of the sponsorship of ongoing imetelstat clinical trials from Janssen to us. In addition, following the effective date of termination of the Collaboration Agreement, we expect Janssen to supply imetelstat to us for up to 24 months during a transition period for clinical manufacturing and such supply will be charged to us at Janssen's cost plus a premium. Our future clinical development plans for imetelstat substantially depend on the timely and comprehensive transition of the imetelstat program from Janssen to us. Delays in completing the transition activities or unwillingness by Janssen to fully perform all of the transition activities will further delay or preclude the clinical development of imetelstat, increase our operating costs and thereby negatively impact our financial results, as well as harm imetelstat's future prospects, any of which could severely and adversely affect our business and business prospects, and might cause us to cease operations.

During the transition period, we remain dependent on Janssen for several key operational development areas. Poor or incomplete performance by Janssen in these areas could severely and adversely affect imetelstat's future value and our business and business prospects, and might cause us to cease operations.

During the transition period, we will remain dependent on Janssen to perform certain activities related to imetelstat, which subjects us to a number of risks, including:

- Janssen may not perform as expected or required by the Collaboration Agreement, and we are not able to control the amount or timing of the resources that Janssen may devote to the transition;

- there may be disputes between us and Janssen that result in the delay of the transition, or the achievement of development, regulatory and commercial objectives, or affect our license to the proprietary rights arising

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under the Collaboration Agreement, which may result in costly litigation or arbitration that diverts our management's attention and resources;

• the manner and timing in which Janssen effects the transition could adversely impact the development of imetelstat;

• failure by Janssen to comply with applicable regulatory guidelines could result in our inability to assume sponsorship responsibility for the IND for imetelstat or to plan for and commence future clinical trials of imetelstat, including the Phase 3 portion of IMerge, or could result in administrative or judicially imposed sanctions on us, including warning letters, civil and criminal penalties, injunctions, product seizures or detention, product recalls, total or partial suspension of manufacturing activities, and the potential refusal to approve any new drug applications;

• our ability to transfer and subsequently maintain the IND for imetelstat and to submit required regulatory reports within required timelines may be compromised if Janssen is not fully cooperative in transferring all data and information from the imetelstat program, including IMbark and IMerge, to us;

• business combinations or significant changes in Janssen's business strategy or failure to apply financial and other resources to the transition may also adversely affect Janssen's ability to perform its obligations related to transition of the imetelstat program to us; and

• Janssen may not properly maintain or defend intellectual property rights arising from the Collaboration Agreement, may use our proprietary information in such a way as to cause disputes that could jeopardize or invalidate our proprietary information or expose us to potential litigation, or may disclose our proprietary information in a manner that could put our intellectual property rights at risk.

The occurrence of any of these events could severely and adversely affect imetelstat's future value and our business and business prospects, and might cause us to cease operations.

RISKS RELATED TO REGULATORY APPROVAL

AND COMMERCIALIZATION OF IMETELSTAT

Maintaining regulatory clearances and approvals to continue the clinical development of imetelstat, and obtaining future regulatory clearances to potentially market imetelstat, in the United States and other countries, is a costly and lengthy process, and we cannot predict when or if regulatory authorities will permit additional imetelstat development or when or if regulatory authorities will approve imetelstat for commercial sale.

Federal, state and local governments in the United States and governments in other countries have significant regulations in place that govern drug research and development and may prevent us from successfully conducting development efforts or commercializing imetelstat. Delays in obtaining regulatory clearances and approvals or limitations in the scope of such clearances or approvals could:

• impede or halt our clinical development activities and plans;

• significantly harm the commercial potential of imetelstat;

• impose additional development costs;

- diminish any competitive advantages that may have been available to us; or

• further delay or preclude any revenue we may receive from the future commercialization of imetelstat, if any.

Before we can seek to obtain regulatory approval for the commercial sale of imetelstat, multiple clinical trials, including larger-scale Phase 3 clinical trials, will need to be conducted to demonstrate if imetelstat is safe and effective for use in a diverse population. Significant additional research, non-clinical testing and clinical testing is required before we can file any application with the FDA or other regulatory authorities for regulatory approval of imetelstat. As such, we do not expect imetelstat to be commercially available for many years, if at all. Our clinical development program for imetelstat may not lead to regulatory approval from the FDA and similar foreign regulatory

authorities if we fail to demonstrate that imetelstat is safe and effective. If imetelstat cannot be developed in potential future clinical trials, including in Phase 3 clinical trials, our business and business prospects would be severely and adversely affected, and we might cease operations. Even if we do successfully complete one or more future clinical trials of

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imetelstat in hematologic myeloid malignancies, including the Phase 3 portion of IMerge, those results are not necessarily predictive of results of future pivotal trials that may be needed before we may submit an NDA to the FDA for the initial or other future indications. We may therefore fail to further develop or commercialize imetelstat.

If our interpretation of safety and efficacy data obtained from non-clinical studies and clinical trials varies from interpretations by the FDA or regulatory authorities in other countries, this would likely further delay, limit or prevent further development and approval of imetelstat. For example, the FDA and regulatory authorities in other countries may require more or different data than what has been generated from our non-clinical studies and previous or ongoing clinical trials, even though protocols for these trials may have been reviewed by FDA and any resulting feedback incorporated. In addition, delays or rejections of regulatory approvals, or limitations in marketing authorizations, may be encountered as a result of changes in the regulatory environment or regulatory policy during the period of product development and/or the period of review of any application for regulatory approval for imetelstat.

The benefit-risk profile of imetelstat will also affect the assessment by the FDA and regulatory authorities in other countries of the drug's cost-effectiveness and/or marketability, which assessment could prevent or limit its approval for marketing and successful commercial use. If regulatory submissions requesting approval to market imetelstat are submitted, the FDA and regulatory authorities in other countries may conclude that the overall benefit-risk profile of imetelstat treatment does not merit approval of imetelstat for marketing or further development for any indication. Any of these events could cause us to halt future development and commercialization of imetelstat, if any, which would severely harm our business and business prospects, and might cause us to cease operations.

Imetelstat must receive all relevant regulatory approvals before it may be marketed in the United States or other countries. Obtaining regulatory approval is a lengthy, expensive and uncertain process. For example, in June 2016, the electorate in the United Kingdom voted in favor of exiting the European Union, and in March 2017, the Government of the United Kingdom initiated the formal procedure of withdrawal from the European Union. Although the impact of the withdrawal of the United Kingdom from the European Union will not be known for some time, this could lead to a period of considerable uncertainty in relation to the regulatory process in Europe, which could result in a delay in the review of regulatory submissions made in Europe by biotechnology and pharmaceutical companies, including potentially by us in the future, and could also lead to less efficient, more expensive, and potentially lengthier regulatory review processes for companies like us, who may seek to obtain regulatory approval for drug products in the European Union or the United Kingdom. Such changes could adversely affect and/or delay our ability to obtain approval of, and market and sell, imetelstat in the United States. In addition, because imetelstat involves the application of new technologies and a new therapeutic approach, it may be subject to substantial additional review by various government regulatory authorities, and, as a result, the process of obtaining regulatory approvals for imetelstat may proceed more slowly than for product candidates based upon more conventional technologies, and any approval that may be received could limit the use of imetelstat. We do not expect imetelstat to be approved for commercial sale for many years, if at all.

Even if the necessary time and resources are committed by us, the required regulatory clearances and approvals may not be obtained for imetelstat. Further, if regulatory clearances and approvals are obtained to commence commercial sales of imetelstat, they may impose significant limitations on the indicated uses or other aspects of the product label for which imetelstat can be marketed. An approval might also be contingent on the performance of costly additional post-marketing clinical trials. Any failure to advance imetelstat to subsequent clinical trials, failure to obtain regulatory approval of imetelstat, or limitations on any regulatory approval that we might receive, could reduce the potential commercial use of imetelstat, and potential market demand for imetelstat and therefore result in decreased revenue for us from any commercialization of imetelstat, any of which would severely and adversely affect our financial results, business and business prospects, and the future of imetelstat, and might cause us to cease operations.

Although orphan drug designation has been granted to imetelstat for the treatment of MF and MDS, these designations may not be maintained, which would eliminate the benefits associated with orphan drug designation, including the potential for market exclusivity, which would likely result in decreased sales revenue from commercialization of imetelstat, if any, and would likely harm our business and business prospects.

The FDA granted orphan drug designation to imetelstat in June 2015 for the treatment of MF and for the treatment of MDS in December 2015, and the European Medicines Agency, or EMA, granted it in December 2015 for the treatment of MF. The designation of imetelstat as an orphan drug does not guarantee that any regulatory authority

will accelerate regulatory review of, or ultimately approve, imetelstat, nor does it limit the ability of any regulatory authority to grant orphan drug designation to product candidates of other companies that treat the same indications as imetelstat prior to imetelstat receiving any exclusive marketing approval.

We may lose orphan drug exclusivity if the FDA or EMA determines that the request for orphan drug designation was materially defective or if we cannot ensure sufficient quantities of imetelstat to meet the needs of patients with MF or MDS.

Even if we maintain orphan drug exclusivity for imetelstat, the exclusivity may not effectively protect imetelstat from competition because different drugs with different active moieties can be approved for the same condition. Even after an orphan drug product is approved, the FDA or EMA can subsequently approve a different drug with the same active moiety for the same condition, if the FDA or EMA concludes that the later drug is safer, more effective, or makes a major contribution to patient care. The occurrence of any of these events could result in decreased sales of imetelstat, should it ever receive marketing approval, and may harm our business and business prospects. In addition, orphan drug designation will neither shorten the development time nor regulatory review time for imetelstat, and does not give imetelstat any advantage in the regulatory review or approval process.

A Fast Track designation by the FDA, such as the Fast Track designation received for imetelstat, does not guarantee approval and may not lead to a faster development, regulatory review or approval process.

In October 2017, the FDA granted fast track designation for the imetelstat clinical development program for the treatment of adult patients with transfusion-dependent anemia due to Low or Intermediate-1 risk MDS who are non-del(5q) and who are refractory or resistant to treatment with an erythropoiesis stimulating agent. Fast track designation provides opportunities for frequent interactions with FDA review staff, as well as eligibility for priority review, if relevant criteria are met, and rolling review. Fast track designation is intended to facilitate and expedite development and review of a New Drug Application to address unmet medical needs in the treatment of serious or life-threatening conditions. However, fast track designation does not accelerate conduct of clinical trials or mean that the regulatory requirements are less stringent, nor does it ensure that imetelstat will receive marketing approval or that approval will be granted within any particular timeframe. In addition, the FDA may withdraw fast track designation if it believes that the designation is no longer supported by data emerging from the imetelstat clinical development program.

Failure to achieve continued compliance with government regulations could delay or halt commercialization of imetelstat.

Approved products and their manufacturers are subject to continual review, and discovery of previously unknown problems with a product or its manufacturer may result in restrictions on the product or manufacturer, including import restrictions, seizure and withdrawal of the product from the market. If approved for commercial sale, future sales of imetelstat will be subject to government regulation related to numerous matters, including the processes of:

- manufacturing;
- advertising and promoting;
- selling and marketing;
- labeling; and
- distribution.

If, and to the extent that, we are unable to comply with these regulations, our ability to earn potential revenue from the commercialization of imetelstat, if any, would be materially and adversely impacted.

Failure to comply with regulatory requirements can result in severe civil and criminal penalties, including but not limited to:

- recall or seizure of products;
- injunctions against the import, manufacture, distribution, sales and/or marketing of products; and

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criminal prosecution.

The imposition of any of these penalties or other commercial limitations would severely and adversely affect our financial results, business and business prospects, and the future of imetelstat, and might cause us to cease operations.

RISKS RELATED TO MANUFACTURING IMETELSTAT

Failure by Janssen to manufacture or provide adequate clinical quantities of imetelstat on a timely basis, or at all, for the period required by the Collaboration Agreement, or our failure to establish a manufacturing supply chain to appropriately and adequately supply imetelstat for future clinical and commercial uses, would result in a further delay in or cessation of clinical trials and a further delay in or our inability to obtain regulatory approvals of imetelstat, and our business and business prospects could be severely harmed, and we could cease operations.

Pursuant to the Collaboration Agreement, Janssen is required to supply imetelstat to us until September 28, 2020 while we are planning to re-establish our own manufacturing supply chain. Consequently, we will remain dependent on Janssen to appropriately supply imetelstat and other clinical trial materials until such date or when we re-establish our own manufacturing supply chain. Thereafter, we will be responsible for the manufacture and supply of imetelstat for future clinical and commercial uses. The process of manufacturing imetelstat is complex and subject to several risks, including:

- the ability to scale-up and attain sufficient production yields with appropriate quality control and quality assurance;
- reliance on third-party manufacturers and suppliers;

- supply chain issues, including the timely availability and shelf life requirements of raw materials and other supplies;
- shortage of qualified personnel; and

- compliance with regulatory requirements, which are less well-defined for oligonucleotide products than for small molecule drugs and vary in each country where imetelstat might be sold or used.

As a result of these and other risks, Janssen may not perform as agreed or may default in its obligations to supply clinical quantities of imetelstat for the period of time required by the Collaboration Agreement, or may fail to deliver the required quantities of imetelstat on a timely basis, or at required or applicable quality standards, which would result in a further delay or cessation of current and potential future clinical trials and otherwise significantly further delay our ability to develop imetelstat, which would severely and adversely affect our business and business prospects, and might cause us to cease operations. In addition, our inability to establish a manufacturing supply chain capable of providing imetelstat for clinical trials and potential future commercial uses following the termination of Janssen's obligation to supply us with imetelstat would further delay or result in a cessation of potential future clinical trials and would further delay or preclude any applications for regulatory approval and therefore further delay or preclude our ability to earn revenue from the commercialization, if any, of imetelstat, which would severely and adversely affect our financial results, business and business prospects, and might cause us to cease operations.

If third parties that manufacture imetelstat fail to perform as needed, then the clinical and commercial supply of imetelstat will be limited, and we may be unable to conduct future clinical trials of imetelstat, including the Phase 3 portion of IMerge, or to commercialize imetelstat in the future.

Following the termination of Janssen's obligation to supply us with imetelstat, we expect to rely solely upon third-party contractors to perform certain process development or other technical and scientific work with respect to imetelstat, as well as to supply starting materials and manufacture drug substance and drug product. We currently have no arrangements with third parties for the manufacture of imetelstat, and the establishment of such arrangements could further delay, perhaps substantially, or preclude our ability to pursue imetelstat development on our own, increase our costs and otherwise negatively affect our financial results, business and business prospects. We may not be able to obtain third-party manufacturers for imetelstat on acceptable terms, or at all. We expect to rely on third-party

contractors to produce and deliver sufficient quantities of imetelstat and other materials to support clinical trials on a timely basis and to comply with applicable regulatory requirements. We will not have direct control over these

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third-party personnel or operations. Reliance on these third-party manufacturers is subject to numerous risks, including:

- being unable to identify suitable third-party manufacturers, because the number of potential manufacturers is limited;
- regulatory authorities may require significant activities to validate and qualify any replacement manufacturer, which could involve new testing and compliance inspections;
- being unable to contract with third-party manufacturers on acceptable terms, or at all;
- the inability of third-party manufacturers to timely formulate and manufacture imetelstat or to produce imetelstat in the quantities or of the quality required to meet clinical and commercial needs;
- decisions by third-party manufacturers to exit the contract manufacturing business during the time required to supply clinical trials or to successfully produce, store and distribute products;
- compliance by third-party manufacturers with current Good Manufacturing Practice, or cGMP, standards mandated by the FDA and state agencies and other government regulations corresponding to foreign regulatory authorities;
- breach or termination of manufacturing contracts;
- inadequate storage at contracted facilities resulting in theft or spoilage;
- capacity limitation and scheduling imetelstat manufacturing activities as a priority in contracted facilities; and
- natural disasters that affect contracted facilities.

Each of these risks could lead to delays or shortages in drug supply, or the inability to manufacture drug supply necessary for non-clinical and clinical activities, and commercialization. For example, manufacturing delays could adversely impact the completion of current clinical trials, such as the extension phase of IMbark and the Phase 2 portion of IMerge, or the commencement of potential future clinical trials, including the Phase 3 portion of IMerge, which could severely and adversely affect our financial results, business and business prospects, and the future of imetelstat, and might cause us to cease operations.

In addition, third-party contractors and/or any other contractors may need to make substantial investments to enable sufficient capacity increases and cost reductions, and to implement those regulatory and compliance standards necessary for successful Phase 3 clinical trials and commercial production of imetelstat. These third-party contractors may not be willing or able to achieve such capacity increases, cost reductions, or regulatory and compliance standards, and even if they do, such achievements may not be at commercially reasonable costs. Changing manufacturers may be prolonged and difficult due to inherent technical complexities and because the number of potential manufacturers is limited. It may be difficult or impossible for us to find a replacement manufacturer on acceptable terms, or at all.

It may not be possible to manufacture imetelstat at costs or scales necessary to conduct clinical trials or potential future commercialization activities.

Oligonucleotides are relatively large molecules produced using complex chemistry, and the cost of manufacturing an oligonucleotide like imetelstat is greater than the cost of making typical small-molecule drugs. Therefore, imetelstat for clinical use is more expensive to manufacture than most other treatments currently available today or that may be available in the future. Similarly, the cost of manufacturing imetelstat for commercial use will need to be significantly lower than current costs in order for imetelstat to become a commercially successful product. We may not be able to achieve sufficient scale increases or cost reductions necessary for successful commercial production of imetelstat. Failure to achieve necessary cost reductions could result in decreased sales, if any, for us, which would materially and adversely affect our financial results, business and business prospects, and the future of imetelstat, and might cause us to cease operations.

RISKS RELATED TO MANAGING OUR GROWTH AND OTHER BUSINESS OPERATIONS

We may be unable to successfully retain or recruit key personnel to support the development and potential future commercialization of imetelstat or to otherwise successfully manage our growth.

Our ability to successfully develop imetelstat in the future and to potentially commercialize imetelstat depends to a significant extent on the skills, experience and efforts of our executive officers and key members of our staff. In addition, we will need to hire a number of senior personnel to re-staff our internal drug development group, as well as to contract with subject matter experts in clinical science, biostatistics, clinical operations, pharmacovigilance, quality systems, manufacturing and regulatory affairs, to enable us to further develop and potentially commercialize imetelstat.

We face intense competition for qualified individuals from numerous pharmaceutical, biopharmaceutical and biotechnology companies, as well as academic and other research institutions, and competition in our geographic region is particularly intense. Termination of the Collaboration Agreement by Janssen, as well as the previous restructurings we implemented, and the uncertainties regarding our future business viability could have an adverse impact on our ability to retain and recruit qualified personnel or we may incur unanticipated inefficiencies caused by our reduced personnel resources. We may also face higher than expected personnel costs in order to attract new management or development personnel, or to maintain our current executive officers and staff. If we are unable to successfully retain, motivate and incentivize our existing personnel, or to attract, assimilate and retain other highly qualified management and senior development personnel in the future on acceptable terms, our ability to further develop imetelstat will be impaired, and our business and the price of our common stock would be adversely impacted.

As our operations potentially expand, we expect that we will need to manage new and additional relationships with various service providers, vendors, suppliers and other third parties, as well as additional office locations, for example, our planned office opening in northern New Jersey. Such potential growth and expansion will require members of our management to assume significant added responsibilities. Our performance in managing any such future growth, if ineffective, could negatively impact our business prospects. We may not successfully manage our anticipated imetelstat development efforts and potential future imetelstat clinical trials, including the Phase 3 portion of IMerge, effectively. If we fail to achieve key development goals, our abilities to grow as a company, and to further develop and commercialize imetelstat, could be prevented or hindered, and our business and business prospects would be severely harmed, which might cause us to cease operations.

We expect imetelstat to remain our sole product candidate for the foreseeable future. If we are unable to successfully develop or commercialize imetelstat, our business and business prospects would be severely harmed, which might cause us to cease operations.

We plan to focus our efforts on the further development of imetelstat in hematologic myeloid malignancies. Accordingly, we do not currently have any plans to engage in any efforts to discover new product candidates or to seek to acquire and/or in-license other oncology products, product candidates, programs or companies in order to diversify our business. Since we do not currently have a discovery function or capabilities, and do not plan to establish such capabilities or to seek to diversify our product candidate portfolio through acquisition and/or in-licensing activity, we will be wholly reliant upon the development of imetelstat, our sole product candidate, for the foreseeable future. If we are unable to successfully develop and commercialize imetelstat, our business and business prospects would be severely harmed, which might cause us to cease operations.

If we are unable to establish potential future collaborative arrangements for imetelstat, we may have to delay, alter or abandon our imetelstat development and commercialization plans.

We intend to develop imetelstat broadly for hematologic malignancies, and to potentially commercialize, market and sell imetelstat by ourselves in the United States. We plan to seek another collaborative partner or partners, at an appropriate time, to assist us in the potential development and commercialization of imetelstat outside the United States, and to provide funding for such activities. We face significant competition in seeking appropriate collaborative partners, and these potential collaborative arrangements are complex and time consuming to negotiate, document and implement. We may not be able to negotiate collaborative arrangements on acceptable terms, or at all. In this regard, collaborative arrangements with third parties may require us to relinquish material rights, including revenue from potential commercialization, on terms that are less attractive than under the Collaboration Agreement we had with Janssen, or to assume material ongoing development obligations that we would have to fund or otherwise support.

In any event, we are unable to predict when, if ever, we will enter into any collaborative arrangements because of the numerous risks and uncertainties associated with establishing collaborative arrangements. Moreover, as a result of the termination of the Collaboration Agreement and the significant uncertainty regarding the future imetelstat development program, potential collaborative partners may be less willing to enter into new collaborative arrangements with us, or may only be willing to do so on terms that are not favorable to us. As a result, we may not be successful in finding a new collaborative partner or partners on favorable terms, if at all. If we are unable to negotiate collaborative arrangements, we may have to:

- curtail the development of imetelstat,
- further delay, alter or abandon the imetelstat development program,
- further delay or abandon its potential commercialization,
- reduce the scope of potential future sales or marketing activities, or
- increase our expenditures and undertake development or commercialization activities at our own expense, which will require substantial additional capital than our current resources.

In order to advance the imetelstat program, including completing the Phase 3 portion of IMerge and potential clinical trials in other indications, as well as potential commercialization activities in the United States, we will need to raise substantial additional capital. In addition, if we elect to increase our expenditures to fund imetelstat development or commercialization activities outside the United States on our own, we will be required to substantially increase our personnel resources and we will need to obtain substantial further capital, which may not be available to us on acceptable terms, or at all. If we do not have sufficient funds, we will not be able to advance the imetelstat program, including completing the Phase 3 portion of IMerge or clinical trials in other indications, or to bring imetelstat to market and generate product revenues. Establishing the infrastructure necessary to further develop, commercialize, market and sell imetelstat worldwide will require substantial resources and may divert the attention of our management and key personnel and negatively impact our imetelstat development or commercialization efforts in the United States.

We currently have no products approved for commercial sale and we have not yet demonstrated an ability to obtain marketing approvals for any product candidates, which makes it difficult to assess our future viability.

We have never derived any revenue from the sales of any products. Our operations to date have been limited to organizing and staffing our company, acquiring, developing and securing our technology, undertaking non-clinical studies and early stage clinical trials of imetelstat and past product candidates that we have subsequently discontinued, and engaging in research and development under collaboration agreements. We have not yet demonstrated an ability to obtain regulatory approvals, formulate and manufacture commercial-scale products, or conduct sales and marketing activities necessary for successful product commercialization. Consequently, for these and other reasons discussed elsewhere in these risk factors, it is difficult to predict our future success and the viability of our business and the imetelstat program.

We may not be able to obtain or maintain sufficient insurance on commercially reasonable terms or with adequate coverage against potential liabilities in order to protect ourselves against product liability claims or claims related to clinical trial conduct.

Our business exposes us to potential product liability and other risks that are inherent in the testing, manufacturing and marketing of human therapeutic and diagnostic products. We may become subject to product liability claims or claims related to clinical trial conduct if the use of imetelstat is alleged to have injured patients, including any injuries alleged to arise from any hepatotoxicity or hemorrhagic event associated with the use of imetelstat. We currently have limited clinical trial liability insurance, and we may not be able to maintain this type of insurance for any clinical trials, including the Phase 3 portion of IMerge, or this type of insurance may become too expensive for us to afford because of the highly risky and uncertain nature of clinical trials generally and the high cost of insurance for our business.

activities. In addition, business liability and product liability insurance are becoming increasingly expensive, particularly for biotechnology and pharmaceutical companies, and the pool of insurers offering insurance coverage to biotechnology and pharmaceutical companies generally is becoming smaller, making it more difficult to obtain insurance for our business activities at a reasonable price, or at all. Being unable to obtain or

maintain product liability, clinical trial liability, or other insurance for our business activities in the future on acceptable terms or with adequate coverage against potential liabilities could have a material adverse effect on our business.

We have been, and may in the future be, involved in securities-related legal actions that are expensive and time consuming. Any securities-related legal actions, if resolved adversely, could harm our business, financial condition, or results of operations.

Securities-related class action lawsuits and/or derivative lawsuits have often been brought against companies, including biotechnology and biopharmaceutical companies, that experience volatility in the market price of their securities. This risk is especially relevant for us because we often experience significant stock price volatility in connection with our product development activities.

We and certain of our officers were named as defendants in two purported class action securities lawsuits filed in the United States District Court for the Northern District of California, or the California District Court, as well as a third securities lawsuit, not styled as a class action, which was transferred to the California District Court. These three cases, or the Class Action Lawsuits, were consolidated for all purposes and settled in July 2017. In connection with the settlement, in April 2017, we paid \$250,000 and our insurance providers paid \$6.0 million to a settlement escrow account to be paid to members of the settlement class, less payment of attorneys' fees and costs to plaintiff's counsel.

The termination of the Collaboration Agreement could also result in litigation arising out of any claims that our stockholders suffered financial losses. The market price of our common stock declined significantly after the announcement on September 27, 2018 of the termination of the Collaboration Agreement, and certain stockholders experienced significant financial losses. Therefore, it is possible that lawsuits will be filed naming us and/or our officers and directors as defendants with respect to the termination of the Collaboration Agreement by Janssen or other matters related to the Collaboration Agreement, future clinical trials of imetelstat, if any, including the Phase 3 portion of IMerge, or other business activities. Monitoring, initiating and defending against legal actions is time-consuming for our management, is likely to be expensive and may detract from our ability to fully focus our internal resources on our business activities. We could be forced to expend significant resources in the settlement or defense of any additional lawsuits, and we may not prevail in such lawsuits. Additionally, we may not be successful in having any such lawsuit dismissed or settled within the limits of our insurance coverage.

We have not established any reserve for any potential liability relating to any lawsuits. It is possible that we could, in the future, incur judgments or enter into settlements of claims for monetary damages. A decision adverse to our interests in any such lawsuit, or in similar or related litigation, could result in the payment of substantial damages, or possibly fines, and could have a material adverse effect on our business, our stock price, cash flow, results of operations and financial condition.

We may be subject to third-party litigation, and such litigation would be costly to defend or pursue and uncertain in its outcome.

Our business may bring us into conflict with our licensees, licensors, or others with whom we have contractual or other business relationships, or with our competitors or others whose interests differ from ours. We may experience employment-related disputes as we seek to expand our personnel resources. If we are unable to resolve those conflicts on terms that are satisfactory to all parties, we may become involved in litigation brought by or against us.

We may face litigation with Janssen arising from or related to the Collaboration Agreement and Janssen's termination of it. Possible disagreements with Janssen could include disagreements regarding the transition of the imetelstat program from Janssen back to us, or the ownership of proprietary rights arising from the work performed by Janssen

under the Collaboration Agreement. We may become involved in performance or other disputes with the CRO we have retained to support our imetelstat clinical development activities, or with other third parties such as service providers, vendors, suppliers or consultants, which could result in a further delay or cessation of current and potential future clinical trials and otherwise significantly further delay our ability to develop imetelstat.

Lawsuits are subject to inherent uncertainties, and defense and disposition costs depend upon many unknown factors. Despite the availability of insurance, we may incur substantial legal fees and costs in connection with litigation. Lawsuits could result in judgments against us that require us to pay damages, enjoin us from certain

activities, or otherwise negatively affect our legal or contractual rights, which could have a significant adverse effect on our business. In addition, the inherent uncertainty of such litigation could lead to increased volatility in our stock price and a decrease in the value of our stockholders' investment in our common stock.

RISKS RELATED TO PROTECTING OUR INTELLECTUAL PROPERTY

Our success and the success of our planned future development of imetelstat will depend on our ability to protect our technologies and imetelstat through patents and other intellectual property rights.

Protection of our proprietary technology is critically important to our business. Our success will depend in part on our ability to obtain, maintain, enforce and extend our patents and maintain trade secrets, both in the United States and in other countries. Our patents may be challenged, invalidated or circumvented, and our patent rights may not provide proprietary protection or competitive advantages to us. In the event that we are unsuccessful in obtaining, maintaining, and enforcing our patents and other intellectual property rights or having our licensors maintain the intellectual property rights we have licensed, the value of our technologies and imetelstat will be adversely affected, and we may not be able to further develop or potentially commercialize imetelstat. Loss or impairment of our intellectual property related to imetelstat might further delay or halt ongoing or potential future clinical trials of imetelstat and any applications for regulatory approval, and therefore further delay or preclude any future development or commercialization of imetelstat by us. Further, if imetelstat is approved for commercial sale, such events could impair our ability to sell imetelstat and therefore result in decreased sales for us. Occurrence of any of these events would materially and adversely affect our financial results, business and business prospects, and the future of imetelstat, and might cause us to cease operations.

Changes in U.S. or foreign patent law or interpretations of such patent laws could diminish the value of our patents in general, thereby impairing our ability to protect our technologies and imetelstat.

The patent positions of pharmaceutical and biopharmaceutical companies, including ours, are highly uncertain and involve complex legal and technical questions. In particular, legal principles for biotechnology and pharmaceutical patents in the United States and in other countries are evolving, and the extent to which we will be able to obtain patent coverage to protect our technologies and imetelstat, or enforce or defend issued patents, is uncertain.

Since the publication of discoveries in scientific or patent literature tends to lag behind actual discoveries by at least several months and sometimes several years, the persons or entities that we name as inventors in our patents and patent applications may not have been the first to invent the inventions disclosed in the patent applications or patents, or the first to file patent applications for these inventions. As a result, we may not be able to obtain patents for discoveries that we otherwise would consider patentable and that we consider to be extremely significant to the future success of imetelstat. Thus, our ability to protect our patentable intellectual property depends, in part, on our ability to be the first to file patent applications with respect to our inventions or inventions that were developed by Janssen under the Collaboration Agreement and to which we have an exclusive license for the further development, commercialization and manufacture of imetelstat. Delay in the filing of a patent application for any purpose, including further development or refinement of an invention, may result in the risk of loss of patent rights.

A number of significant changes to U.S. patent law occurred when the Leahy-Smith America Invents Act, or the AIA, was signed into law on September 16, 2011. These include provisions that affect the way patent applications are examined and may affect patent litigation. Many of the substantive changes to patent law associated with the AIA, and in particular, the first to file provisions, became effective on March 16, 2013. For example, the AIA limits where a patentee may file a patent infringement suit. The AIA and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

U.S. court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. For example, on June 13, 2013, the U.S. Supreme Court, or the Court, issued a decision in *Association for Molecular Pathology v. Myriad Genetics, Inc.* holding that claims to isolated genomic DNA were not patentable subject matter, but claims to complementary DNA, or cDNA, molecules were patentable subject matter. On March 20, 2012, in *Mayo Collaborative Services, DBA Mayo Medical Laboratories, et al. v. Prometheus Laboratories, Inc.*, the Court held that several claims drawn to measuring drug metabolite levels from patient samples and correlating them to drug doses were not patentable subject matter. In addition, court rulings in cases such as *BRCA1- & BRCA2-Based Hereditary Cancer Test Patent Litig.* and *Promega Corp. v. Life Technologies Corp.* have also narrowed the scope of patent protection available in certain circumstances. In addition

to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events may have created uncertainty with respect to the value of certain patents we have previously obtained or in-licensed.

In addition, in June 2016, the electorate of the United Kingdom voted to exit the European Union, and in March 2017 the Government of the United Kingdom initiated the formal procedure of withdrawal from the European Union. While the exit of the United Kingdom from the European Union is planned, the exact timing of the withdrawal and the resulting effect of withdrawal will not be known for some time, which could lead to a period of considerable uncertainty relating to our ability to obtain and maintain Supplementary Protection Certificates of our products based on our United Kingdom patents and our ability to establish and maintain European trademarks in the United Kingdom.

In 2012, the European Union Patent Package, or EU Patent Package, regulations were passed with the goal of providing for a single pan-European Unity Patent, or UP, and a new European Unified Patent Court, or UPC, for litigation of European patents. Once established, the UPC would have jurisdiction over traditional European patents and new UPs in the United Kingdom and all Contracting Member States of the European Union. However, political activity in the United Kingdom and a legal challenge in Germany has delayed ratification of the EU Patent Package in these countries. There have been many delays in the implementation of the EU Patent Package, and further delays may occur. When the EU Patent Package is ratified and in effect, all European patents, including those issued prior to ratification, would by default automatically fall under the jurisdiction of the UPC and allow for the possibility of obtaining pan-European injunctions. Under the EU Patent Package as currently proposed, once the UPC is established, patent holders are permitted to “opt out” of the UPC on a patent-by-patent basis, although the time permitted for this opt-out is not yet known. Owners of traditional European Patent applications who receive notice of grant after the EU Patent Package is ratified could validate the patent nationally, and file an opt-out demand. The EU Patent Package may increase the uncertainties and costs surrounding the enforcement or defense of our issued European patents. The full impact on future European patent filing strategy and the enforcement or defense of our issued European patents in member states and/or the UPC is not known.

Depending on decisions by the U.S. federal courts, the U.S. Patent and Trademark Office, or the Patent Office, and similar authorities in foreign jurisdictions, the interpretation of laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents. Occurrence of these events and/or significant impairment of our imetelstat patent rights would severely and adversely affect our financial results, business and business prospects, and the future of imetelstat, which might cause us to cease operations.

Challenges to our owned or licensed patent rights would result in costly and time-consuming legal proceedings that could prevent or limit development of imetelstat.

Our patents or those patent rights we have licensed, including patent rights that we may seek with respect to inventions made by Janssen under the Collaboration Agreement, may be challenged through administrative or judicial proceedings, which could result in the loss of important patent rights. For example, where more than one party seeks U.S. patent protection for the same technology, the Patent Office may declare an interference proceeding in order to ascertain the party to which the patent should be issued. Patent interferences are typically complex, highly contested legal proceedings, subject to appeal. They are usually expensive and prolonged, and can cause significant delay in the issuance of patents. Our pending patent applications or our issued patents, or those we have licensed and may license from others, including Janssen, may be drawn into interference proceedings or be challenged through post-grant review procedures or litigation, any of which could delay or prevent the issuance of patents, or result in the loss of issued patent rights.

Under the AIA, interference proceedings between patent applications filed on or after March 16, 2013 have been replaced with other types of proceedings, including derivation proceedings. The AIA also includes post-grant review

procedures subjecting U.S. patents to post-grant review procedures similar to European oppositions, such as inter partes review, or IPR, covered business method post-grant reviews and other post-grant reviews. This applies to all of our U.S. patents and those we have licensed and may license from others, including Janssen, even those issued before March 16, 2013. Because of a lower evidentiary standard necessary to invalidate a patent claim in Patent Office proceedings compared to the evidentiary standard in U.S. federal court, a third party could potentially provide evidence in a Patent Office proceeding sufficient for the Patent Office to hold a claim invalid even though the same evidence would be insufficient to invalidate the claim if first presented in a district court action. Accordingly, a third

party could attempt to use the Patent Office procedures to invalidate patent claims that would not have been invalidated if first challenged by the third party as a defendant in a district court action. U.S. patents owned or licensed by us may therefore be subject to post-grant review procedures, as well as other forms of review and re-examination. In addition, the IPR process under the AIA permits any person, whether they are accused of infringing the patent at issue or not, to challenge the validity of certain patents. As a result, entities associated with hedge funds have challenged valuable pharmaceutical patents through the IPR process. Significant impairment of our imetelstat patent rights would severely and adversely affect our financial results, business and business prospects, and the future of imetelstat, which might cause us to cease operations.

Certain jurisdictions, such as Europe, New Zealand and Australia, permit oppositions to be filed against granted patents or patents proposed to be granted. Because we may in the future seek to commercialize imetelstat internationally, securing both proprietary protection and freedom to operate outside of the United States is important to our business. Opposition proceedings require significant time and costs, and if we are unsuccessful or are unable to commit these types of resources to protect our imetelstat patent rights, we could lose our patent rights and we could be prevented or limited in the development and commercialization of imetelstat.

As more groups become engaged in scientific research and product development in the areas of telomerase biology and hematologic malignancies, the risk of our patents, or patents that we have in-licensed, being challenged through patent interferences, derivation proceedings, IPRs, post-grant proceedings, oppositions, re-examinations, litigation or other means will likely increase. For example, litigation may arise as a result of our decision to enforce our patent rights against third parties. Challenges to our patents through these procedures would be extremely expensive and time-consuming, even if the outcome was favorable to us. An adverse outcome in a patent dispute could severely harm our ability to further develop or commercialize imetelstat, or could otherwise have a material adverse effect on our business, and might cause us to cease operations, by:

- causing us to lose patent rights in the relevant jurisdiction(s);
- subjecting us to litigation, or otherwise preventing us from commercializing imetelstat in the relevant jurisdiction(s);
- requiring us to obtain licenses to the disputed patents;
- forcing us to cease using the disputed technology; or
- requiring us to develop or obtain alternative technologies.

We may be subject to infringement claims that are costly to defend, and such claims may limit our ability to use disputed technologies and prevent us from pursuing research, development, manufacturing or commercialization of imetelstat.

The commercial success of imetelstat will depend upon our ability to research, develop, manufacture, market and sell imetelstat without infringing or otherwise violating the intellectual property and other proprietary rights of third parties. There is considerable intellectual property litigation in the biotechnology and pharmaceutical industries, and many pharmaceutical companies, including potential competitors, have substantial patent portfolios. In the event our technologies infringe the rights of others or require the use of discoveries and technologies controlled by third parties, we may be prevented from pursuing research, development, manufacturing or commercialization of imetelstat, or may be required to obtain licenses to those patents or other proprietary rights or develop or obtain alternative technologies. For example, we are aware that certain third parties have or may be prosecuting patents and patent estates that may relate to imetelstat, and while we believe these patents will expire before imetelstat is commercialized and/or that these patents are invalid and/or would not be infringed by the manufacture, use or sale of imetelstat, it is possible that the owner(s) of these patents will assert claims against us in the future. If that were to occur, we would need to obtain unblocking licenses from such third parties, develop alternative non-infringing technologies, which we may not be able to do at an acceptable cost or on acceptable terms, or at all, or cease the development of imetelstat. In addition, while Janssen has terminated the Collaboration Agreement, we are still subject to indemnification obligations to Janssen under the Collaboration Agreement, including with respect to claims of third party patent infringement.

Since we cannot be aware of all intellectual property rights potentially relating to imetelstat and its uses, we do not know with certainty that imetelstat, or the intended commercialization thereof, does not and will not infringe or otherwise violate any third party's intellectual property. Any infringement claims against us would likely be expensive to resolve, and the cost of any unblocking license that we could be required to obtain is unpredictable and could be significant. If we are unable to resolve an infringement claim successfully, we could be subject to an injunction that would prevent us from commercializing imetelstat and could also require us to pay substantial damages. In addition to infringement claims, in the future we may also be subject to other claims relating to intellectual property, such as claims that we have misappropriated the trade secrets of third parties. Provided that we are successful in continuing the development of imetelstat, we expect to see more efforts by others to obtain patents that are positioned to cover imetelstat. Our success therefore depends significantly on our ability to operate without infringing patents and the proprietary rights of others.

We may become aware of discoveries and technologies controlled by third parties that are advantageous or necessary to further develop or manufacture imetelstat. For example, as we transition the imetelstat program from Janssen to us, we may learn of changes to the imetelstat manufacturing process made by Janssen which would require us to obtain licenses to third party intellectual property rights. Under such circumstances, we may initiate negotiations for licenses to other technologies as the need or opportunity arises. We may not be able to obtain a license to a technology required for the research, development, manufacture or commercialization of imetelstat on commercially favorable terms, or at all, or such licenses may be terminated on certain grounds, including as a result of our failure to comply with the obligations under such licenses. If we do not obtain a necessary license or if such a license is terminated, we may need to redesign such technologies or obtain rights to alternative technologies, which may not be possible, and even if possible, could cause further delays in the development efforts for imetelstat and could increase the development and/or production costs of imetelstat. In cases where we are unable to license necessary technologies, we could be subject to litigation and prevented from researching, developing, manufacturing or commercializing imetelstat which would materially and adversely impact our business. Failure by us to obtain rights to alternative technologies or a license to any technology that may be required to research, develop, manufacture or commercialize imetelstat would further delay potential future clinical trials of imetelstat and any applications for regulatory approval, impair our ability to sell imetelstat and therefore result in decreased sales of imetelstat for us. Occurrence of any of these events would materially and adversely affect our business, and might cause us to cease operations.

We may become involved in disputes with past or future collaborator(s), including Janssen, over intellectual property inventorship or ownership, and publications by us, or by investigators, scientific consultants, research collaborators or others could impair our ability to obtain patent protection or protect our proprietary information, which, in either case, could have a significant impact on our business.

Inventions discovered under research, material transfer or other collaborative agreements, including our Collaboration Agreement with Janssen which was terminated effective September 28, 2018, may become jointly owned by us and the other party to such agreements in some cases, and may be the exclusive property of either party in other cases. Under some circumstances, it may be difficult to determine who invents and owns a particular invention, or whether it is jointly owned, and disputes can arise regarding inventorship and ownership of those inventions. These disputes could be costly and time-consuming, and an unfavorable outcome could have a significant adverse effect on our business if we were not able to protect our license rights to these inventions. In addition, clinical trial investigators, scientific consultants and research collaborators generally have contractual rights to publish data and other proprietary information, subject to review by the trial sponsor. Publications by us, or by investigators, scientific consultants, previous employees, research collaborators or others, either with permission or in contravention of the terms of their agreements with us or with Janssen or otherwise, may impair our ability to obtain patent protection or protect proprietary information which would have a material adverse effect on our business, and might cause us to cease operations.

Much of the information and know-how that is critical to our business is not patentable, and we may not be able to prevent others from obtaining this information and establishing competitive enterprises.

We sometimes rely on trade secrets to protect our proprietary technology, especially in circumstances in which we believe patent protection is not appropriate or available. We attempt to protect our proprietary technology in part by confidentiality agreements with our employees, consultants, collaborators and contractors. We cannot provide assurance that these agreements will not be breached, that we would have adequate remedies for any breach, or that our trade secrets will not otherwise become known or be independently discovered by competitors, any of which would harm our business significantly.

In May 2016, the Defend Trade Secrets Act of 2016, or the DTSA, was enacted, providing a federal cause of action for misappropriation of trade secrets. Under the DTSA, an employer may not collect enhanced damages or attorney fees from an employee or contractor in a trade secret dispute brought under the DTSA, unless certain advanced provisions are observed. We cannot provide assurance that our existing agreements with employees and contractors contain notice provisions that would enable us to seek enhanced damages or attorneys' fees in the event of any dispute for misappropriation of trade secrets brought under the DTSA.

Significant disruptions of information technology systems, including cloud-based systems, or breaches of data security could adversely affect our business.

Our business is increasingly dependent on critical, complex and interdependent information technology systems, including cloud-based systems, to support business processes as well as internal and external communications. Our computer and information technology systems, and those of our collaborators, service providers and contractors, are potentially vulnerable to breakdown, malicious intrusion, malware, computer viruses, natural disasters, terrorism, war, and telecommunication and electrical failures that may result in damage to or the impairment of key business processes, or the loss or corruption of confidential information, including intellectual property, proprietary business information and personal information. Such disruptions and breaches of security could have a material adverse effect on our business, financial condition and results of operations. The costs to us to mitigate network security problems, bugs, viruses, worms, malicious software programs and security vulnerabilities could be significant, and while we have implemented security measures to protect our data security and information technology systems, our efforts to address these problems may not be successful, and these problems could result in unexpected interruptions, delays, cessation of service and other harm to our business and our competitive position. In addition, we will rely on our collaborators, service providers and contractors to establish and maintain appropriate information technology systems and data security protections. However, except for contractual duties and obligations, we have limited ability to control their actions related to such matters. If such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our imetelstat development program. For example, the loss of clinical study data from completed, ongoing or planned clinical trials could result in delays in potential regulatory approval efforts and significantly increase our costs to recover or reproduce the data.

In addition, our computer and information technology systems, as well as those of our collaborators, service providers and contractors, are potentially vulnerable to data security breaches, whether by employees, contractors, consultants, malware, phishing attacks, or other cyber-attacks, that may expose confidential information, intellectual property, proprietary business information or personal information to unauthorized persons. If a data security breach affects our systems or those of third parties upon which we rely, corrupts our data or results in the unauthorized disclosure or release of personally identifiable information by our collaborators, service providers, contractors or us, our reputation could be materially damaged and we could be subject to significant fines, increased costs or loss of revenue. In addition, such a breach may require notification to governmental agencies, supervisory bodies, credit reporting agencies, the media or individuals pursuant to various federal, state and foreign data protection, privacy and security laws, regulations and guidelines, if applicable. These may include state breach notification laws, and the EU General Data Protection Regulation (EU) 2016/679, or GDPR. Accordingly, a data security breach or privacy violation that leads to unauthorized access to, disclosure or modification of personal information (including protected health information), that prevents access to personal information or materially compromises the privacy, security, or confidentiality of the personal information, could result in fines, increased costs or loss of revenue as a result of:

- harm to our reputation;
- fines or penalties imposed on us by regulatory authorities;
- additional compliance obligations or enforcement measures under federal, state or foreign laws;
- remediation and corrective action we undertake as required by law or as otherwise necessary;
- litigation and potential civil or criminal liability; and

requirements to verify the accuracy of affected data.

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If we are unable to prevent such data security breaches or privacy violations or implement satisfactory remedial measures, our operations could be disrupted, and we may suffer loss of reputation, financial loss and other regulatory penalties because of lost or misappropriated information, including sensitive patient data. In addition, these breaches and other inappropriate access can be difficult to detect, and any delay in identifying them may lead to increased harm of the type described above. Moreover, the prevalent use of mobile devices that access confidential information increases the risk of data security breaches, which could lead to the loss of confidential information, trade secrets or other intellectual property. While we have implemented security measures to protect our computer and information technology systems, because the techniques used to obtain unauthorized access, disable or degrade service, or sabotage systems change frequently, become more sophisticated, and often are not recognized until launched against a target, we or our collaborators, service providers or contractors may be unable to anticipate these techniques or to implement adequate preventative measures. In addition, failure to maintain effective internal accounting controls related to data security breaches and cybersecurity in general could impact our ability to produce timely and accurate financial statements and could subject us to regulatory scrutiny.

Changes in and failures to comply with U.S. and foreign privacy and data protection laws, regulations and standards may adversely affect our business, operations and financial performance.

We are subject to or affected by numerous federal, state and foreign laws and regulations, as well as regulatory guidance, governing the collection, use, disclosure, retention, and security of personal data, such as information that we collect about patients and healthcare providers in connection with clinical trials in the United States and abroad. The global data protection landscape is rapidly evolving, and implementation standards and enforcement practices are likely to remain uncertain for the foreseeable future. This evolution may create uncertainty in our business, affect our or our collaborators', service providers' and contractors' ability to operate in certain jurisdictions or to collect, store, transfer use and share personal information, necessitate the acceptance of more onerous obligations in our contracts, result in liability or impose additional costs on us. The cost of compliance with these laws, regulations and standards is high and is likely to increase in the future. Any failure or perceived failure by us or our collaborators, service providers and contractors to comply with federal, state or foreign laws or regulation, our internal policies and procedures or our contracts governing processing of personal information could result in negative publicity, diversion of management time and effort and proceedings against us by governmental entities or others. In many jurisdictions, enforcement actions and consequences for noncompliance are rising.

In the United States, California enacted the California Consumer Privacy Act (CCPA) on June 28, 2018, which takes effect on January 1, 2020. The CCPA gives California residents expanded rights to access and delete their personal information, opt out of certain personal information sharing, and receive detailed information about how their personal information is used. The CCPA provides for civil penalties for violations, as well as a private right of action for data breaches that is expected to increase data breach litigation. The CCPA may increase our compliance costs and potential liability. Some observers have noted that the CCPA could mark the beginning of a trend toward more stringent privacy legislation in the United States, which could increase our potential liability and adversely affect our business.

Our operations abroad may also be subject to increased scrutiny or attention from data protection authorities. Many countries in these regions have established or are in the process of establishing privacy and data security legal frameworks with which we, our collaborators, service providers and contractors must comply. For example, the EU has adopted the GDPR, which went into effect in May 2018 and introduces strict requirements for processing the personal information of EU subjects, including clinical trial data. The GDPR has and will continue to increase compliance burdens on us, including by mandating potentially burdensome documentation requirements and granting certain rights to individuals to control how we collect, use, disclose, retain and process information about them. The processing of sensitive personal data, such as physical health condition, may impose heightened compliance burdens under the GDPR and is a topic of active interest among foreign regulators. In addition, the GDPR provides for more

robust regulatory enforcement and fines of up to €20 million or 4% of the annual global revenue of the noncompliant company, whichever is greater. As we expand into other foreign countries and jurisdictions, we may be subject to additional laws and regulations that may affect how we conduct business.

RISKS RELATED TO OUR FINANCIAL POSITION AND NEED FOR ADDITIONAL FINANCING

Our failure to obtain additional capital when needed could force us to further delay, reduce or eliminate development of imetelstat, including the Phase 3 portion of IMerge, or our potential future imetelstat commercialization efforts, which would severely and adversely affect our financial results, business and business prospects, and might cause us to cease operations.

Successful drug development and commercialization requires significant amounts of capital. We will require substantial additional capital in order to further advance the imetelstat program, including conducting the research and development and clinical and regulatory activities necessary to bring imetelstat to market, such as completion of the Phase 3 portion of IMerge and potential clinical trials in other indications, and to establish sales and marketing capabilities to commercialize imetelstat in the United States on our own, if regulatory approval is granted. Because the outcome of any clinical activities and/or regulatory approval process is highly uncertain, we cannot reasonably estimate whether any development activities we may undertake will succeed, and we may never recoup our investment in any imetelstat development, which would adversely affect our financial condition and our business and business prospects, and might cause us to cease operations. Our future capital requirements are difficult to forecast and will depend on many factors, including:

- the accuracy of the assumptions underlying our estimates for our capital needs;
- the progress, timing, magnitude, scope and costs of clinical development, manufacturing and commercialization of imetelstat, including the number of indications being pursued, subject to clearances and approvals by the FDA and other regulatory authorities;
- the scope, progress, duration, results and costs of current and future clinical trials, including the Phase 3 portion of IMerge, as well as non-clinical studies and assessments, of imetelstat;
- the costs, timing and outcomes of regulatory reviews or other regulatory actions related to imetelstat, including obtaining regulatory clearances and approvals in the United States and in other countries;
- the costs of preparing, filing and prosecuting patent applications and maintaining, enforcing and defending intellectual property-related claims;
- the costs of manufacturing imetelstat, including our ability to meaningfully reduce those manufacturing costs;
- the costs of multiple third-party vendors and service providers, including our CRO, to support the development, manufacturing and commercialization of imetelstat;
- our ability to establish, enforce and maintain collaborative or other strategic arrangements for research, development, clinical testing, manufacturing, commercialization and marketing of imetelstat;
- our ability to successfully market and sell imetelstat, if imetelstat receives future regulatory approval or clearance, in the United States and other countries;
- our need to hire additional qualified employees and consultants to support the development and commercialization of imetelstat;
- the costs and timing of building a U.S. sales force to market and sell imetelstat, should it receive regulatory clearance;
- the sales price for imetelstat;
 - the availability of coverage and adequate third-party reimbursement for imetelstat;
- the extent and scope of our general and administrative expenses, including expenses associated with potential future litigation; and
- the costs of maintaining and operating facilities in California and New Jersey, including higher expenses for travel, telecommunications and administrative oversight.

As a result of the termination of the Collaboration Agreement effective September 28, 2018, we are responsible for funding all clinical development, manufacturing, intellectual property maintenance and commercial activities for imetelstat. In order to further advance the imetelstat program, including completion of the Phase 3 portion of IMerge and potential clinical trials in other indications, we will need to raise substantial additional capital or establish additional collaborative arrangements with third-party collaborative partners, which may not be possible. In addition, as a result of the termination of the Collaboration Agreement, we will not receive any further milestone payments or royalties from Janssen for the development or sale of imetelstat, including any clinical development milestones. If we are unable to raise additional capital or establish alternative collaborative arrangements with third-party collaborative partners for imetelstat, the development of imetelstat may be further delayed, altered or abandoned, which might cause us to cease operations. Additional financing through public or private equity financings, including pursuant to our 2018 Sales Agreement with B. Riley FBR, capital lease transactions or other financing sources may not be available on acceptable terms, or at all. We may raise equity capital at a stock price or on other terms that could result in substantial dilution of ownership for our stockholders. The receptivity of the public and private equity markets to proposed financings is substantially affected by the general economic, market and political climate and by other factors which are unpredictable and over which we have no control. In this regard, continued volatility and instability in the global financial markets and political climate could adversely affect our ability to raise additional funds through financings and the terms upon which we may raise those funds.

In addition, we may seek additional capital due to market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interests of our stockholders may be diluted, and the terms may include liquidation or other preferences that materially and adversely affect the rights of our stockholders. Debt financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions such as incurring additional debt, making capital expenditures or declaring dividends. If we raise additional funds through alliance, collaboration or licensing arrangements with third parties, we may have to relinquish valuable rights to imetelstat or our technologies or grant licenses on terms that are not favorable to us.

We cannot assure you that our existing capital resources, future interest income, and potential future sales of our common stock, including under our 2018 Sales Agreement with B. Riley FBR, will be sufficient to fund our operating plans. We will need additional funds to meet operational needs and capital requirements to advance the imetelstat program in clinical development and potential commercialization, and our need for additional funds may arise sooner than planned. If adequate funds are not available on a timely basis, if at all, we may be unable to pursue further development or potential commercialization of imetelstat, which could adversely affect our business.

We currently have no source of product revenue and may never become consistently profitable.

Although we were profitable in 2015 due to the recognition of revenue in connection with the upfront payment from Janssen under the Collaboration Agreement, we have otherwise never been profitable and have incurred operating losses every year since our operations began in 1990. We will not receive any future milestone-based or royalty payments from Janssen relating to imetelstat, nor will Janssen share the cost of ongoing or future clinical trials of imetelstat or the costs for patents that were licensed to them under the terminated Collaboration Agreement, after September 28, 2018. We expect to incur significant additional operating losses and, as we assume responsibility for imetelstat clinical development activities, our operating losses are likely to substantially increase. As of December 31, 2018, our accumulated deficit was approximately \$1.01 billion. Losses have resulted principally from costs incurred in connection with our research and development activities and from general and administrative costs associated with our operations.

Substantially all of our revenues to date have been payments under collaborative agreements and milestones, royalties and other revenues from our licensing arrangements. With the termination of the Collaboration Agreement effective

September 28, 2018, we have no ongoing collaborative agreements related to imetelstat. Any revenues generated from our remaining licensing agreements related to our telomerase technology are expected to be minimal, and will be insufficient to sustain our operations. Our telomerase-related licensing revenues declined significantly in 2018 due to the expiration of the patents underlying such technology, and are expected to be eliminated later in 2019. We have no current plans to enter into any new corporate collaboration, partnership or license agreements that result in revenues, and our remaining telomerase-related license agreements may be terminated by the other parties to such licenses, or expire with the underlying patents.

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We also expect to experience increased negative cash flow for the foreseeable future as we fund our operations and assume full payment responsibility for imetelstat clinical development activities. This will result in decreases in our working capital, total assets and stockholders' equity. Further, we may be unable to replenish our working capital by future financings. We will need to generate significant revenues to achieve consistent future profitability. We may never achieve consistent future profitability. Even if we do become profitable in the future, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to achieve consistent future profitability could negatively impact the market price of our common stock and our ability to sustain operations.

The comprehensive U.S. tax reform bill passed in 2017 could adversely affect our business and financial condition.

On December 22, 2017, President Trump signed into law legislation, known as the Tax Cuts and Jobs Act of 2017, that significantly revised the Internal Revenue Code of 1986, as amended, or the Code. The legislation, among other things, contained significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%; reduction in the percentage of allowable expenses eligible for orphan drug credit purposes; limitation of the deduction for net operating losses to 80% of current year taxable income and elimination of net operating loss carrybacks; immediate deductions for certain new investments instead of deductions for depreciation expense over time; and modifying or repealing many business deductions and credits. Notwithstanding the reduction in the corporate income tax rate, the overall long-term impact of the federal tax law changes are uncertain and our business and financial condition could be adversely affected. In addition, it is uncertain if and to what extent various states will conform to the federal tax law changes. The impact of this tax reform on holders of our common stock is also uncertain and could be adverse. We urge our stockholders to consult with their legal and tax advisors with respect to this legislation and the potential tax consequences of investing in or holding our common stock.

Our ability to use our net operating loss carryforwards and certain other tax attributes may be limited.

Our net operating loss carryforwards could expire unused and be unavailable to offset future income tax liabilities. Under the 2017 federal income tax law changes, federal net operating losses incurred in 2018 and in future years may be carried forward indefinitely, but the deductibility of such federal net operating losses is limited. It is uncertain if and to what extent various states will conform to the federal tax law changes. In addition, under Section 382 of the Code, and corresponding provisions of state law, if a corporation undergoes an "ownership change," generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period, the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes (such as research tax credits) to offset its post-change taxable income or taxes may be limited. Changes in our stock ownership, some of which are outside of our control, may have resulted or could in the future result in an ownership change. If a limitation were to apply, utilization of a portion of our domestic net operating loss and tax credit carryforwards could be limited in future periods. In addition, a portion of the carryforwards may expire before being available to reduce future income tax liabilities which could adversely impact our financial position.

RISKS RELATED TO OUR COMMON STOCK AND FINANCIAL REPORTING

Historically, our stock price has been extremely volatile.

Historically, our stock price has been extremely volatile. Between January 1, 2009 and December 31, 2018, our stock has traded as high as \$8.73 per share and as low as \$0.86 per share. Between January 1, 2016 and December 31, 2018, the price has ranged between a high of \$6.99 per share and a low of \$0.95 per share. The significant market price fluctuations of our common stock have been due to and may in the future be influenced by a variety of factors, including:

termination of the Collaboration Agreement by Janssen in September 2018;
announcements regarding the research and development of imetelstat, or results of, further delays in, discontinuation of, or further modifications or refinements to any clinical trials of imetelstat for any reason, including as a result of the failure to successfully transition the imetelstat program to us by Janssen, or our inability, for any reason, to successfully continue the development of imetelstat after any such transfer;