Bio-Path Announces Clinical Update to Interim Analysis of Phase 2 Prexigebersen Trial in Acute Myeloid Leukemia

Interim Data Update from Phase 2 Study Demonstrates Meaningful Clinical Improvement with Excellent Patient Safety Profile

Company Provides Clinical Development Plan with Pathways to Registration

HOUSTON – March 6, 2019 – Bio-Path Holdings, Inc., (NASDAQ: BPTH), a biotechnology company leveraging its proprietary DNAbilize® antisense RNAi nanoparticle technology to develop a portfolio of targeted nucleic acid cancer drugs, today announces a clinical update to the previously reported interim analysis from the Phase 2 trial of prexigebersen (BP1001) for the treatment of acute myeloid leukemia (AML) and provides its plans for the compound's clinical development moving forward toward registration.

The open-label Phase 2 study in Stage 1 evaluated the efficacy and safety of prexigebersen in conjunction with low dose cytarabine (LDAC), a therapeutic regimen well-established in treatment of AML patients who cannot or elect not to be treated with more intensive chemotherapy. The primary objective of the study is to determine whether the combination of prexigebersen and LDAC provides greater efficacy than would be expected with LDAC alone in a *de novo* patient population. Subsequently, Stage 2 of the study added a second cohort that is evaluating the efficacy and safety of prexigebersen in conjunction with Decitabine in addition to the cohort evaluating prexigebersen in conjunction with LDAC.

In April 2018, Bio-Path completed an initial interim analysis of 17 evaluable patients from Stage 1 of the Phase 2 study. These results showed a promising safety and efficacy profile with 47% of patients having a response comprised of four complete response (CR) patients, including one CR_i (complete response with incomplete hematologic recovery) and four patients with stable disease. Recently, the data from the 17 evaluable patients was updated, and following a meeting with the principal investigators of the study, those results now show that the efficacy profile has improved to where 11 (65%) of the 17 evaluable patients had a response, including five (29%) who achieved CR (including one CR_i) and one morphologic leukemia free state (MLFS), and six stable disease responses, including two patients who had greater than a 50% reduction in bone marrow blasts. Importantly, through investigation by the principal investigators, it was observed that 68% of these patients were secondary AML patients, an extremely difficult class to treat.

The efficacy data from the 17 evaluable patients was very favorable in this challenging population compared to the reported CR (complete response), CRp (complete response with incomplete platelet recovery), and CRi rates with LDAC treatment alone of 7-13%[1] in this

patient population. Additionally, a study of newly approved Venetoclax plus LDAC in these newly diagnosed patients reported a 42% CR/CRh (complete response with incomplete hematologic response) response rate; however, this study had only 46% secondary AML compared to 68% in the Bio-Path 17-patient interim analysis.

"These updated interim data from Stage 1 of our Phase 2 study of prexigebersen in *de novo* AML patients give strong evidence of the safety and efficacy profile of our lead compound and underscore its potential to provide meaningful treatment improvement in this difficult-to-treat patient population," said Peter Nielsen, President and Chief Executive Officer of Bio-Path. "We were particularly pleased with these results, especially when you consider that the large percentage of these patients are secondary AML patients. The CR/CRp/CRi rate for LDAC treatment alone for the class of patients in this study was benchmarked at 7-13%[1], whereas prexigebersen treatment with LDAC is currently showing a 29% CR/CRi/MLFS rate, with a highly favorable safety profile."

"Prexigebersen with its efficacy and safety profile, is an ideal combination candidate with frontline therapy. Our aim is to match prexigebersen with the leading frontline therapies to improve treatment options for patients. Consequently, we maintain an in-depth knowledge of all new therapies and therapies in development. As the treatment landscape evolves, we continue to nimbly respond to those advances and the plans for our registration-directed clinical development program for prexigebersen as a treatment for AML reflects these changes," concluded Mr. Nielsen.

The recent approval of the frontline therapy Venetoclax provides an opportunity for combining prexigebersen with the combination Venetoclax plus Decitabine for the treatment of *de novo* AML patients. Venetoclax is a drug whose activity is against the anti-apoptotic protein Bcl-2 based on neutralizing the protein's BH3 domain. It is also an approved treatment in chronic lymphocytic leukemia (CLL) patients; however, with the exception of some patients treated with allogeneic hematopoietic cell transplantation (HCT), disease relapse invariably occurs, often times due to BH3 domain mutation over time. Bio-Path's BP1002 is a drug candidate that targets the Bcl-2 protein, just as Venetoclax. However, BP1002 activity is based on blocking the Bcl-2 messenger RNA, and not the BH3 domain. As a result, Bio-Path believes that BP1002 could provide an alternative to Venetoclax CLL patients who have relapsed. Likewise, Bio-Path believes there will be AML patient relapses from Venetoclax treatments, representing an additional opportunity for Bio-Path to treat those patients with BP1002.

As a result, Bio-Path intends to file for registration of BP1002 for the treatment of Venetoclax relapses in both CLL patients and AML patients. The planned modification of the Company's Phase 2 clinical program in AML to include Venetoclax combination treatment with prexigebersen will give Bio-Path early experience with treating Bcl-2 driven anti-apoptosis in these patients.

Registrational Clinical Development Program

After treating nearly 70 patients, Bio-Path believes it now has a plan with definable paths to registration. Results to date have shown prexigebersen, with its efficacy and safety profile, to be an ideal combination candidate with frontline therapy. The Principal Investigators of the Phase 2 study and Bio-Path's Scientific Advisory Board helped prepare the revised clinical program for prexigebersen in AML. The new registration-directed plan is as follows:

- Amend the existing Stage 2 prexigebersen + Decitabine Phase 2 AML cohort in untreated *de novo* patients to add untreated high risk myelodysplastic syndrome (MDS) patients. High risk MDS patients are typically treated with Hypomethylating agents alone and the combination treatment may benefit these patients.
- Cancel the Stage 2 prexigebersen + LDAC Phase 2 AML cohort in untreated *de novo* patients. Although Bio-Path has had good success with prexigebersen + LDAC, there is a strong preference by oncologists for Decitabine over LDAC as the combination therapy drug partner in treating these patients.
- Amend the existing Phase 2 protocol to add a cohort of prexigebersen in combination with Decitabine in refractory/relapsed AML patients. This is based on the Company's experience in this setting, including the Phase 1b safety segment combination treatment in refractory/relapsed patients. Refractory/relapsed high risk MDS patients will be included in this cohort.
- Preclinical efficacy studies are underway for prexigebersen + Decitabine + Venetoclax triple combination to confirm the incremental efficacy benefit of the triple combination.
- Amend the protocol of the Phase 2 trial to perform a small safety assessment of the triple combination prexigebersen + Decitabine + Venetoclax in the refractory/relapsed AML plus high risk MDS patient cohort.
- Following a successful safety assessment, initiate a registration-directed cohort of the trial by adding Venetoclax to the prexigebersen + Decitabine combination treatment of refractory/relapsed AML plus high risk MDS patients.
- Amend the protocol of the Phase 2 trial to initiate a Prexigebersen + Decitabine + Venetoclax registration-directed trial for untreated AML and high risk MDS patients, to determine if more durable responses and longer survival is observed compared to patients treated with the Decitabine + Venetoclax combination.

The result from these transformational steps will be two registration-directed cohorts of Bio-Path's Phase 2 clinical trial in AML, both studying the triple combination prexigebersen + Decitabine + Venetoclax but in two separate patient populations, including untreated AML plus untreated high risk MDS patients, and refractory/relapsed AML plus refractory/relapsed high risk MDS patients. Bio-Path expects that many of the Venetoclax patients will relapse and that Bio-Path second drug candidate BP1002 targeted to Bcl-2 can replace Venetoclax enabling continued patient treatment with the triple combination. We expect this to result in a third registration-directed clinical program, specifically for BP1002 in Venetoclax treatment failures.

[1] Heiblig, Mediterr J Hematol 2016; Kantarjian, J Clin Oncol 2012; Dohner, Blood 2014.

About Bio-Path Holdings, Inc.

Bio-Path is a biotechnology company developing DNAbilize®, a novel technology that has yielded a pipeline of RNAi nanoparticle drugs that can be administered with a simple intravenous transfusion. Bio-Path's lead product candidate, prexigebersen (BP1001, targeting the Grb2 protein), is in a Phase 2 study for blood cancers and in preclinical studies for solid tumors. This is followed by BP1002, targeting the Bcl-2 protein, which the company anticipates entering into clinical studies where it will be evaluated in lymphoma and solid tumors.

For more information, please visit the Company's website at http://www.biopathholdings.com.

Forward-Looking Statements

This press release contains forward-looking statements that are made pursuant to the safe harbor provisions of the federal securities laws. These statements are based on management's current expectations and accordingly are subject to uncertainty and changes in circumstances. Any express or implied statements contained in this press release that are not statements of historical fact may be deemed to be forward-looking statements. Any statements that are not historical facts contained in this release are forward-looking statements that involve risks and uncertainties, including Bio-Path's ability to raise needed additional capital on a timely basis in order for it to continue its operations, Bio-Path's ability to have success in the clinical development of its technologies, the timing of enrollment and release of data in such clinical studies and the accuracy of such data, limited patient populations of early stage clinical studies and the possibility that results from later stage clinical trials with much larger patient populations may not be consistent with earlier stage clinical trials, the maintenance of intellectual property rights, risks relating to maintaining Bio-Path's listing on the Nasdaq Capital Market and such other risks which are identified in Bio-Path's most recent Annual Report on Form 10- K, in any subsequent quarterly reports on Form 10-Q and in other reports that Bio-Path files with the Securities and Exchange Commission from time to time. These documents are available on request from Bio-Path Holdings or at www.sec.gov. Bio-Path disclaims any intention or obligation to update or revise any forward-looking statements, whether as a result of new information, future events or otherwise

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