



Corporate Presentation February 2016

“A New Paradigm in Antisense Drug Delivery”



Forward Looking Statements

This Presentation contains forward-looking statements with respect to business conducted by Bio-Path Holdings, Inc. By their nature, forward-looking statements and forecasts involve risks and uncertainties because they relate to events and depend on circumstances that will occur in the future.

The Company does not undertake to update any forward-looking statements. There are a number of factors that could cause actual results and developments to differ materially and investors should use their own judgment to evaluate risks.

Overview

- ❖ About Bio-Path Holdings
- ❖ Antisense and DNAbilize™ Technology
- ❖ BP1001 (Liposomal Grb2) in the clinic
- ❖ Future plans and milestones
- ❖ Financial snapshot



Bio-Path Holdings

- ❖ Oncology-focused pharmaceutical development company located in Houston, TX
- ❖ Established in 2008 with technology licensed from the University of Texas MD Anderson Cancer Center
- ❖ Listed on NASDAQ in March 2014 as BPTH
- ❖ Breakthrough science and cancer drugs
 - DNAbilize™ Technology is our proprietary antisense and delivery system that solves the antisense industry dilemma
 - Demonstrated ability to deliver antisense DNA into target cells and down-regulate the protein
- ❖ First drug targeted to Grb2, BP1001, is in Phase I/II trials for AML; Ph+ CML
 - Received Orphan Drug Designation for AML from the FDA
 - Results of Phase I/II combination testing very encouraging
- ❖ Triple negative and inflammatory breast cancer in preclinical stage
- ❖ Second target to Bcl2 (BP1002) in preparation for a clinical trial for follicular lymphoma

Core Organization

Peter Nielsen

Co-Founder, President, Chief Executive Officer and Chief Financial Officer

- Officer and Director since founding Company in 2007

Ulrich Mueller, PhD

Chief Operating Officer

- Previously Vice President at the Fred Hutchinson Cancer Research Center
- Former Managing Director Office of Technology Commercialization at MD Anderson

Ana M. Tari, PhD & MBA

Director, Preclinical Operations & Research

- Key member of the research team that developed our liposomal delivery technology

Tara Sadeghi, M.P.H.

Director, Clinical Operations

- More than 24 years of drug development and clinical operations experience across all phases of clinical development (Phases I through III)

Suzanne Kennedy, PhD

Director, Corporate Development

- More than 15 years of marketing, business development, and research & development experience in the biotech industry

Focus

- Clinical team added to manage clinical trials and place new candidates into an IND, clinical trial
- Expanding preclinical research and manufacturing capabilities

Scientific Advisory Board

Jorge Cortes, M.D.

Chairman

- M.D. from la Facultad de Medicina, Universidad Nacional Autónoma de México
- Jane and John Justin Distinguished Chair in Leukemia Research, Chief of the AML and CML sections, and Deputy Chair of the Department of Leukemia at The University of Texas MDACC
- Has consulted leading pharmaceutical companies such as AstraZeneca on development of prenyltransferase inhibitors, GlaxoSmithKline on the use of topotecan in MDS and CMML, and Rhône-Poulenc Rorer on the use of PEG-Asparaginase in adult ALL

Amy P. Sing, M.D.

Member, Bio-Path's Board of Directors

- M.D. from the Stanford University School of Medicine
- Currently Senior Director of Medical Affairs at Genomic Health, Inc.
- Former Senior Medical Director at Genentech, Inc., had integral role in the Avastin™ program
- Former Senior Director of Medical and Regulatory Affairs at Seattle Genetics

Recruiting additional members

DNAbilize™ Antisense DNA: A Targeted Method for Treating Disease

- ❖ **Antisense** - molecules that interfere with the process of producing proteins inside cells (RNAi)
 - Does not use a toxic agent to kill cells, but instead blocks production of proteins
 - Advantage of specificity because it targets the disease-causing protein
- ❖ **No toxicity** - In numerous animal studies or human patients in BP1001 clinical trial
 - DNAbilize™ liposome structure is similar to the cellular membrane
 - P-ethoxy DNA does not activate complement or inhibit the clotting cascade
- ❖ **Systemic treatment** - I.V. delivery to the main organs via blood flow
- ❖ **High cellular uptake** - liposome structure is similar to the cellular membrane
- ❖ **Microscopic-sized liposomes** - enable penetration into tumors for delivery of drug
- ❖ **Proven target inhibition** - demonstrated that DNAbilize™ method inhibits target protein, proving delivery technology works

Technology Platform: DNAbilize™

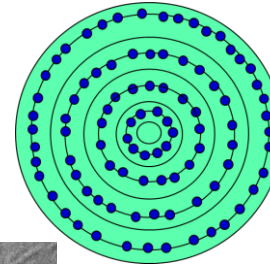
DNAbilize™ Technology is our proprietary antisense and delivery system that solves the antisense industry dilemma

- ➔ Overcomes limitations of toxicity from DNA modification and the lipid delivery
- ➔ Overcomes inability to deliver systemically; targets outside of the liver now treatable
- ➔ Overcomes inability to dose at therapeutic levels

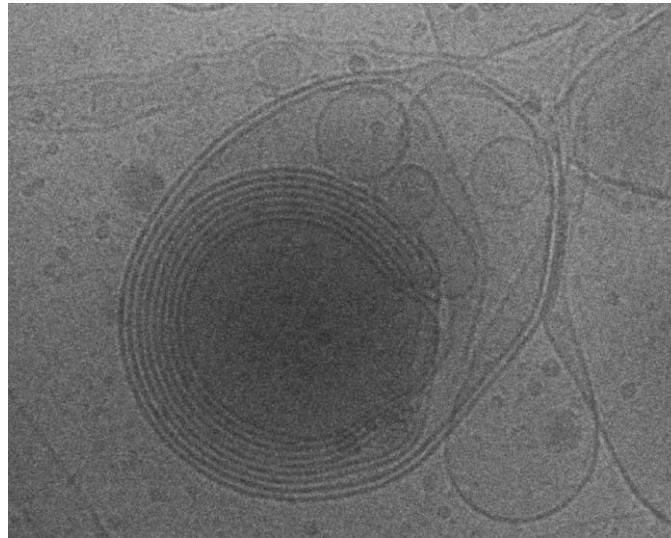
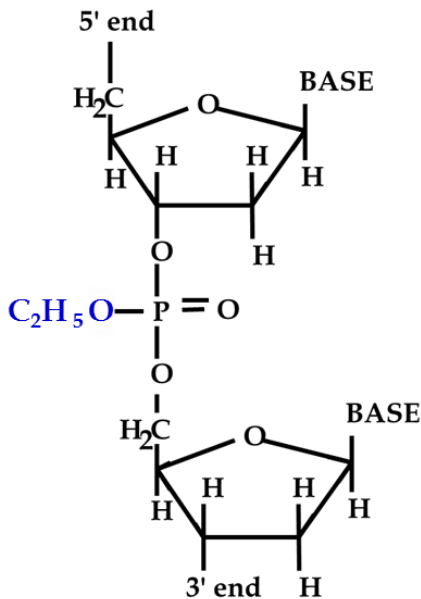
P-ethoxy DNA



Neutral Liposome



DNAbilize™



DNAbilize™ Lead Target and Indications: Grb2 and Myeloid Leukemia

BP1001 is an antisense DNA to the bridging protein Grb2

- ❖ Ideal target for myeloid leukemia because it shuts down the Ras pathway in receptor activated myeloid cells without exerting adverse effects on Ras signaling through other channels

No toxicity *in vivo* to date

- ❖ Acute Myeloid Leukemia: high unmet need for a fragile patient population
 - Average age at diagnosis \geq 60 years old
 - > 20,000 new cases a year
 - > 10,000 deaths a year
 - Patients typically cannot tolerate induction chemotherapy. Patients have lower response rates and decreased survival

No new AML treatments have been approved in 25 years (Idarubicin)

BP1001 in Clinical Testing for Acute and Chronic Myeloid Leukemia

- ❖ Phase I monotherapy clinical trial in relapsed/refractory patients
 - First 6 cohorts completed
 - Preliminary results show drug has been well tolerated in patients with AML, CML, and MDS with no signs of toxicity and signs of anti-leukemia activity
 - BP1001 received Orphan Drug Designation for AML in 2015

- ❖ Safety segment of Phase II (Phase Ib) trial for AML in combination with frontline therapy LDAC
 - Cohort 7 (60 mg/m²) completed with 2 of 3 patients achieving **complete remission**
 - Cohort 8 (90 mg/m²) fully enrolled
 - Phase II efficacy trial planned to start early 2016

- ❖ Phase II safety segment for combination therapy in CML to begin early 2016

- ❖ Additional indications for solid tumors in preclinical development

Summary of Phase I Monotherapy and Ib Combination Therapy Clinical Trial Results for BP1001

- ❖ AML, CML, ALL & MDS Patients Refractory or Resistant to Current Therapies
- ❖ Dose escalating, treatment cycle 8 doses over 4 weeks
- ❖ Results through cohort 6 (90 mg/m²)
 - Patients averaged **6 prior therapies**
 - **15 of 20 evaluable patients' blasts demonstrated anti-leukemia activity**
 - 8 patients stabilized for extended treatment
 - Drug was **well tolerated**
- ❖ Of the 18 evaluable with circulating blasts, **83% had a response to the drug**
- ❖ Cohort 7 (Phase II safety segment)
 - LDAC + 60 mg/m² BP1001
 - **2 patients achieved complete remission**

Patients	Diagnosis	Peripheral or bone marrow (BM)* Blast %		Reason Discontinued	Cycles Completed
		Baseline	Nadir		
01	CML	93	82	DLT	<1
06	AML	15	2	PD	5
07	MDS	8*	4*	PD	5
010**	AML	23*	10*	PD	1
011	CML	24	7	PD	1
014**	AML	33	5	PD	1
015	AML	51	31	PD	1
020	AML	76	5	PD	1
021	AML	71	43	PD	2
022	AML	1	0	PD	2
023	MDS	NE*	NE*	WD	1***
024	MDS	0*	0*	PD	5
025	AML	10	3	PD	2
026	AML	16	none	PD	1
027	AML	93	92	PD	1
028	AML	96	none	PD	1
029	AML	33	7	PD	1
030	AML	51	17	PD	1
031	AML	17	NE	PD	1
032	AML	24	NE	PD	2
034	AML	66	ND	PD	1
★ 035	AML	17	2	CR	1
037	AML	25	25	PD	1
★ 038	AML	23	2	CR	5

Cohort 7 {

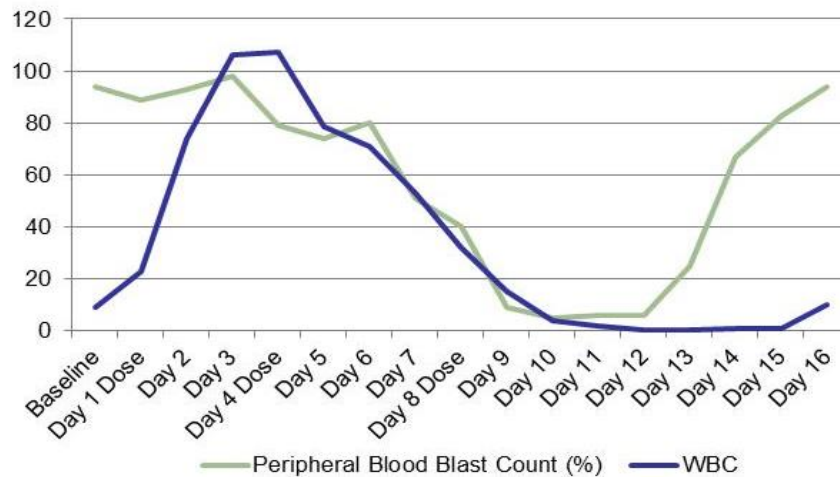
★ Complete remission

Response to Treatment for Blast Crisis CML and HIV+ AML

❖ **Patient 002:** 32 year-old, Hispanic male with myeloid blast crisis of CML

❖ Prior therapies consist of:

- Gleevec
- Dasatinib
- Nilotinib
- DCC-2036
- Cytarabine/Fludarabine/
- Dasatinib/Gemtuzumab
- PHA-739358
- Clofarabine/Dasatinib

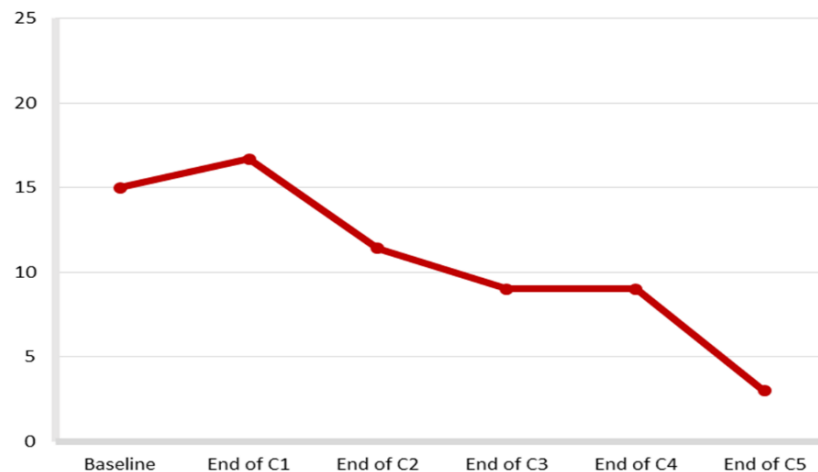


❖ Patient taken off therapy, CNS disease was assumed (not confirmed) and intrathecal Ara-C was administered - patient succumbed due to disease progression prior to going back on treatment

❖ **Extended Treatment: Patient 006**

- ❖ 54 year-old HIV+ male with AML transformed from Jak2 positive Polycythemia Vera
- ❖ 3 patients showed improvement and/or stable disease, received 5 treatment cycles over 5 months
- ❖ Patient 006 achieved stable disease and marked reduction in peripheral blasts

Peripheral Blood % Blast



BP1001 Downregulates Grb2 and Downstream pErk Proteins in Target Cells

- Grb2 levels decreased 50% in 11 of 13 samples by end of treatment (EOT)
- pErk levels decreased an average of 52% in 7 of 13 samples by EOT.

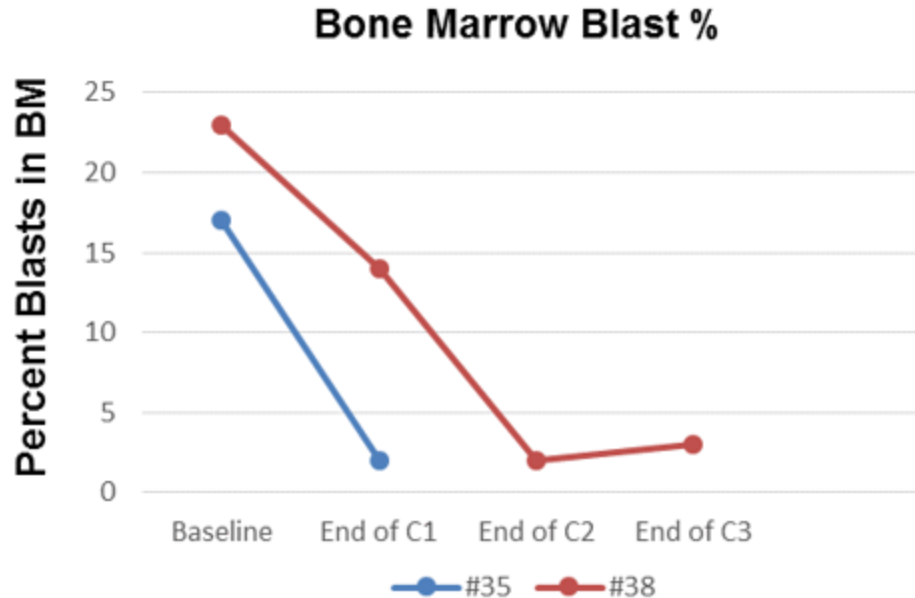
Subject Number	Grb2 Decrease (Day 15)	pErk Decrease (Day 15)	Grb2 Decrease (EOT)	pErk Decrease (EOT)
022	0	0	57	0
023	0	3	28	45
024	56	28	47	35
025	63	82	54	91
026	47	0	0	0
027	NS ¹	NS ¹	34	27
028	0	0	30	54
029	57	51	65 ²	0 ²
030	54	55	43	47
031	0	0	0	0
032	85	54	91	63
033	13	13	53	2
034	42	42	40	0

Results of Phase II Cohort 7 Combination LDAC + BP1001 Therapy

- ❖ Safety evaluation of combination low-dose cytarabine (LDAC) with BP1001 in refractory and relapsed patients
- ❖ Three (3) evaluable patients were treated twice a week for 4 weeks with 60 mg/m² of BP1001 and LDAC
- ❖ Total of 8 doses in combination with the standard regimen of LDAC
 - Two out of three patients achieved ***complete remission***
- ❖ Cohort 8 patients being treated with 90 mg/m² of BP1001 in combination with frontline LDAC is ongoing

Results were consistent with previous cohorts, showing BP1001 to be safe and well tolerated, with significant anti-leukemia activity

% Blasts in Bone Marrow for Patients in Phase II Cohort 7



★ Complete remission

Patients	Diagnosis	Peripheral or bone marrow (BM)* Blast %		Cycles Completed
		Baseline	Nadir	
★ 035	AML	17	2	1
037	AML	25	25	1
★ 038	AML	23	2	5

Plans For Phase II Efficacy Trial for BP1001 AML Combination Therapy

- ❖ Currently completing the safety segment of the Phase II (Phase Ib) trial evaluating 2 cohorts at 2 dose levels, 3 patients per cohort, to test for any potential negative synergies of using BP1001 together with frontline therapy (LDAC)
 - Planning to have approximately 54 patients with an interim analysis after 19 patients
 - If successful, the trial will be rolled into a pivotal trial for accelerated approval
 - Conducted at leading cancer centers in the U.S., including the MD Anderson Cancer Center
 - Primary endpoint for the study is the number of patients who achieve complete remission

- ❖ Phase II trial in CML starting in Q1 of 2016

Recent Accomplishments

- ❖ Phase I monotherapy clinical trial completed through six cohorts with very promising results
 - 34 patients enrolled
 - Well tolerated drug with no toxicity
 - 18 evaluable patients with circulating blasts, 83% had a response
- ❖ BP1001 inhibits disease-causing protein in human patients with blood cancers
- ❖ BP1001 being developed for solid tumors including triple negative and inflammatory breast cancers
- ❖ Phase II safety segment (Phase Ib) dosing for the combination therapy for BP1001 near completion with patients achieving complete remission
- ❖ IND package for BP1002 (Liposomal Bcl2) in preparation to begin a clinical trial
- ❖ Promising new targets in preclinical development
- ❖ \$25 million ATM financing in place

Upcoming Milestones

- ❖ Completion of safety segment of Phase II (Phase Ib) clinical trial for AML
- ❖ Expanding pipeline with new target drug candidates (lymphoma, pancreatic, brain)
- ❖ New drug indication outside of cancer
- ❖ Value propositions for 2016:
 - Enrollment of first 19 patients in Phase II with an interim analysis to be completed with potential for switch to registration trial for accelerated approval
 - Initiation of safety segment for Phase II (Phase Ib) in CML for combination therapy
 - Demonstrated effectiveness of delivery technology (broad drug development, licensing implications)
 - Continued new manufacturing and target IP

Clinical Pipeline

	TARGET INDICATIONS	PRECLINICAL	IND	PHASE I	PHASE II
Liposomal Grb-2 (BP1001)	*AML	→			
Liposomal Grb-2 (BP1001)	CML	→			
Liposomal Grb-2 (BP1001)	Breast Cancer	→			
Liposomal Grb-2 (BP1001)	Lymphoma, Colon, Thyroid and Head & Neck Cancers	→			
Liposomal Bcl-2 (BP1002)	Follicular Lymphoma	→			
DNAbilize™ Technology			READY TO OUT-LICENSE		

*BP1001 for AML has received orphan drug designation from the U.S. FDA

Investment Summary

Broad Vision, Focused Strategy

- Solving the antisense drug delivery challenge with DNAbilize™ Technology
- World's leading cancer institution, MD Anderson, is significant shareholder

Novel Mechanism of Action

- Eliminates the obstacles to successful antisense drug therapeutics
- Systemic delivery enables treatment of diseases not possible with other technologies
- No toxicity allows for development of drugs for hard to treat diseases and unmet needs among fragile populations

Significant Market Opportunity

- Initial two drug candidates have market potential of \$4+ billion
- Expanding pipeline with new targets and indications
- Actively pursuing licensing opportunities for DNAbilize™

Financial Snapshot

Ticker: NASDAQ: BPTH

Shares: 89.8 million shares outstanding Sept 30, 2015

Market Cap: Approximately \$125 million

Capital Raised: \$30.6 million

Cash: \$9.9 million as of Sept 30, 2015

Burn rate: Approximately \$1,000,000 per quarter excluding the clinical trial costs

Financing: \$25 million ATM program in place