



Corporate Presentation
January 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

Investment Highlights

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

- Liposomal delivery technology distributes antisense drugs throughout the human body by simple intravenous infusion

Versatile Delivery Technology

- Ability to apply delivery technology template to almost any protein target and expand outside cancer
- Numerous new drug candidates and partners identified for development

Significant Market Opportunity

- Initial two drug candidates have market potential of \$4+ billion
- Expanding pipeline with potential for licensing

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, PhD

Director, Clinical Operations

- Over 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

- Over 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.

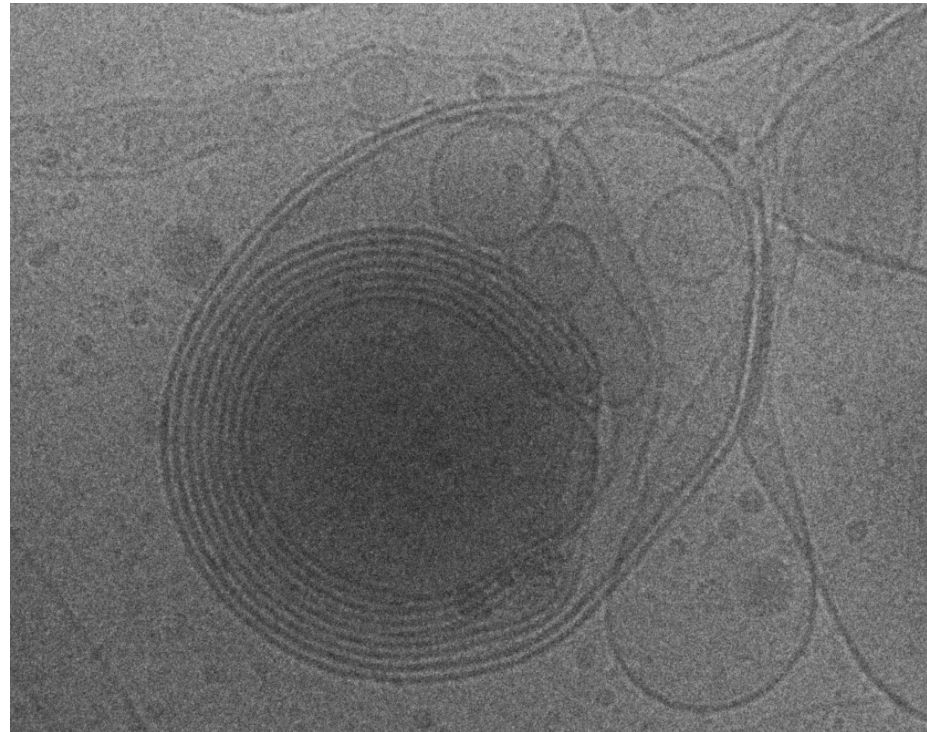
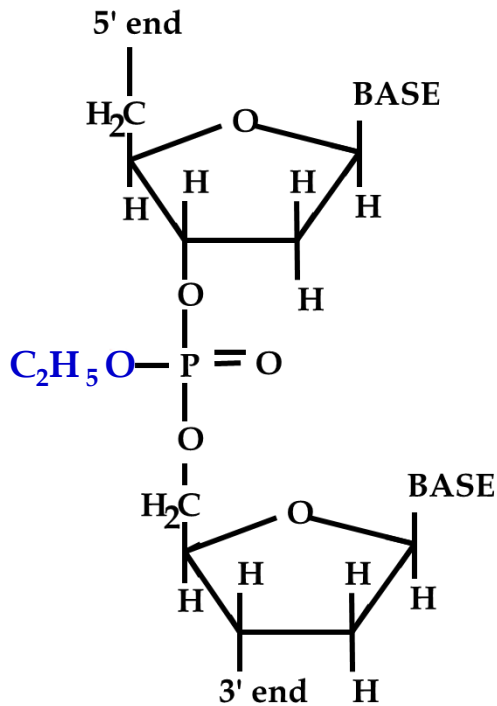
Member, Bio-Path's Board of Directors

- 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 an integral role in the Avastin™ program
- Former Senior Director of Medical and Regulatory Affairs at Seattle Genetics

Recruiting additional members

DNAbilize™ Technology

- ❖ P-ethoxy DNA incorporate into the neutral liposomes' hydrophobic multi-lamellar structure in contrast to electrostatic interactions in cationic lipid delivery methods.
- ❖ Neutral liposomes avoid toxic effects of cationic liposomes on cell membranes by avoiding nonspecific interactions with plasma proteins and negatively charged cell surfaces.



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

BP1001 in Clinical Testing

- ❖ Grb2 is a protein that bridges activated tyrosine kinases to the Ras signaling pathway
- ❖ Phase I clinical trial:
 - 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
- ❖ Safety segment of Phase II (Phase Ib) trial for Acute Myeloid Leukemia (AML) in combination with frontline therapy ongoing
 - Phase II efficacy trial planned to start early 2016
 - Phase II trial in Chronic Myelogenous Leukemia (CML) planned
- ❖ Additional indications in triple negative and inflammatory breast cancers being developed and in other solid tumors

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 treatments
- 2 patients (010 and 014) transient improvement leukemia cutis lesions
- Drug was **well-tolerated**
- ❖ Of the 18 evaluable with circulating blasts, 83% had a response to the drug
- ❖ Cohort 7 (Phase II safety segment) receiving LDAC + 60 mg/m² BP1001, 2 patients, 035 and 038 achieved complete remission.

Patients	Diagnosis	Peripheral or bone marrow (BM)* Blast %			Cycles Completed
		Baseline	Nadir	Off-Tx	
01	CML	93	82	97	<1
06	AML	15	2	5	5
07	MDS	8*	4*	6*	5
010**	AML	23*	10*	10*	1
011	CML	24	7	50	1
014**	AML	33	5	21	1
015	AML	51	31	72	1
020	AML	76	5	23	1
021	AML	71	43	74	2
022	AML	1	0	2	2
023	MDS	NE*	NE*	NE*	1***
024	MDS	0*	0*	2*	5
025	AML	10	3	19	2
026	AML	16	none	80	1
027	AML	93	92	97	1
028	AML	96	none	98	1
029	AML	33	7	27	1
030	AML	51	17	84	1
031	AML	17	NE	17	1
032	AML	24	NE	40	2
034	AML	66	ND	ND	1
★035	AML	17	2	ND	1
037	AML	25	25	ND	1
★038	AML	23	2	ongoing	4

Cohort 7 {

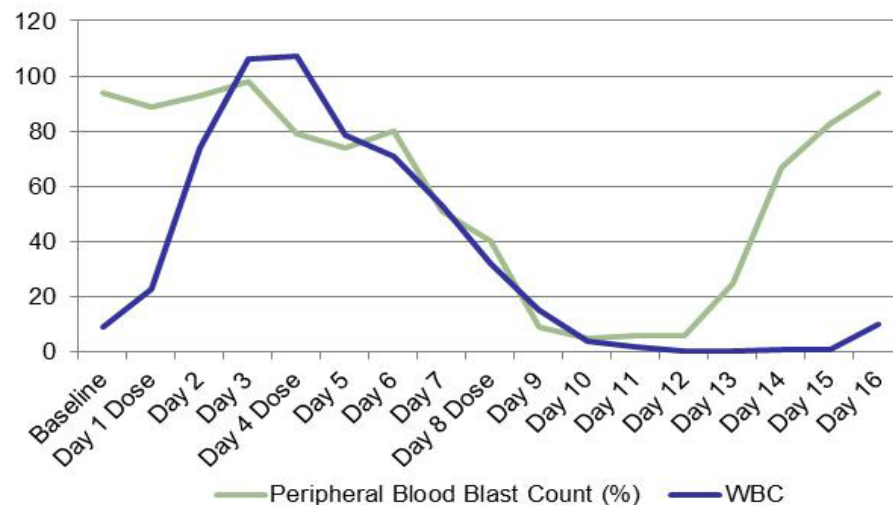
★ Complete remission

Response to Treatment for Patients 002 and 006

❖ **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

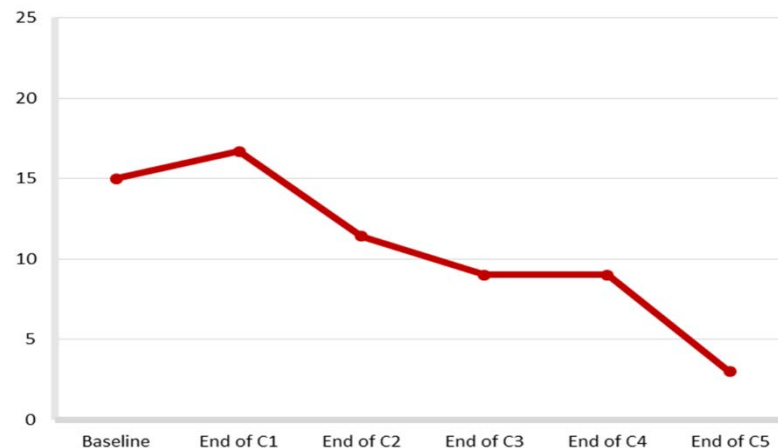
❖ **Extended Treatment: Patient 006**

❖ 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



% Decrease of Target Grb2 Protein and pErk protein

- 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

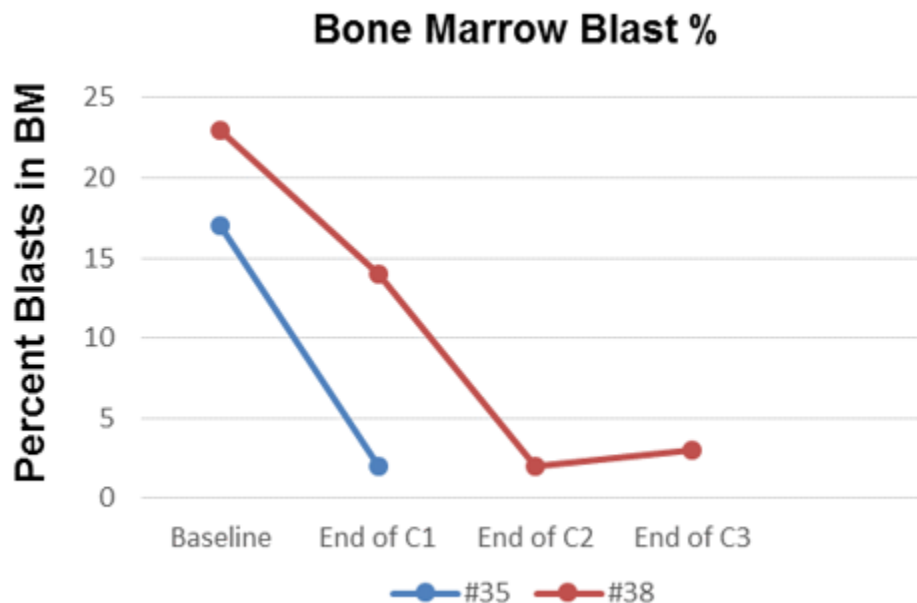
Results of Phase II Cohort 7 Combination LDAC + BP1001 Therapy

- ❖ 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, well tolerated with significant anti-leukemia activity

Percent Blasts in Bone Marrow for Patients in Safety Study

Phase II Cohort 7



Patients	Diagnosis	Peripheral or bone marrow (BM) Blast %			Cycles Completed
		Baseline	Nadir	Off-Tx	
035	AML	17	2	yes	1
037	AML	25	25	yes	1
038	AML	23	2	ongoing	4

Plans For Phase II Clinical Trials of BP1001

- ❖ The proposed clinical program is to evaluate BP1001 in AML in a Phase II clinical trial in combination with frontline 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
- ❖ Phase II efficacy trial:
 - 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
 - Expected to be conducted at leading cancer centers in the US, 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
 - 8 of 21 evaluable patients stabilized for additional treatment
- ❖ BP1001 inhibits disease-causing protein in human patients with blood cancers
- ❖ BP1001 being developed for triple negative and inflammatory breast cancers
- ❖ Significant corporate development in 2015:
 - 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
- ❖ Continued pre-clinical evaluation of BP1001 for inflammatory and triple negative breast cancer and if successful, rapid deployment into a Phase I clinical trial
- ❖ Exciting new drug candidates coming from our business development team
 - 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)
 - Expanding pipeline and collaborations on new and creative drug candidates
 - 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 and ALL	▶			
Liposomal Grb-2 (BP1001)	Breast Cancer	▶			
Liposomal Grb-2 (BP1001)	Lymphoma, Colon, Thyroid and Head & Neck Cancers	▶			
Liposomal Bcl-2 (BP1002)	Follicular Lymphoma	▶			

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

Financial Snapshot

Ticker: NASDAQ: BPTH

Shares: 89.8M shares outstanding Sept 30, 2015

Market Cap: approximately \$130 MM

Capital Raised: \$30.6 MM

Cash: \$9.9 MM 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