



Company Presentation March 2018

“A new path in DNA-powered medicine”



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.

DNAbilize[®] Technology

- ✓ Enables the development and delivery of systemic antisense RNAi nanoparticle treatments for a broad spectrum of cancers including hematological malignancies, solid tumors, as well as diseases outside of cancer
 - Based on single agent therapeutic treatments, DNAbilize[®] is the only technology that has shown no evidence of toxicity while producing therapeutic effect
 - Has the ability to address hard to treat diseases and unmet clinical needs in fragile populations
- ✓ *DNAbilize[®] is **NOT** siRNA and is **NOT** like other antisense technology that have associated toxicity and no delivery*

Investment Highlights

Broad Vision, Focused Strategy

- Advanced oligonucleotide therapeutics with high efficiency delivery
- New composition and methods of use patents to cover DNAbilize® technology solely owned by Bio-Path

Clinical Stage Pipeline

- Lead candidate prexigebersen in Phase II for AML and CML
- Second drug candidate being reviewed with the FDA for IND to start Phase I

Novel Mechanism of Action

- Demonstrated ability to down-regulate target protein in diseased cells in systemic diseases
- Lack of toxicity allows for treatment of fragile populations

Robust Preclinical Pipeline

- Applications and development in other cancers including solid tumors and indications outside of cancer

Validating Academic Collaborations

- MD Anderson developing clinical and preclinical pipeline in pancreatic cancer and solid tumors including advanced ovarian cancer
- Thomas Jefferson University establishing DNAbilize® Technology for glioblastoma immunotherapy

DNAbilize® Antisense and Technology Profile: A Targeted Method for Treating Disease

- **Antisense RNAi** - a process of interfering with cellular protein production using DNA in a nanoparticle
 - Does not use a toxic agent to kill cells, but blocks production of proteins
 - Advantage of specificity because it targets the disease-causing protein
- **No toxicity** - In numerous animal studies and human patients in prexigebersen clinical trial
 - DNAbilize™ liposome structure is similar to the cellular membrane
 - P-ethoxy DNA does not induce hepatotoxicity or thrombocytopenia
- **Systemic treatment** - I.V. delivery to the main organs via blood flow
- **High cellular uptake** - liposome structure is similar to the cellular membrane
- **Nanoparticle liposomes** - enable penetration into tumors for delivery of drug
- **Proven target inhibition** - demonstrated that DNAbilize® method inhibits target protein, proving delivery technology works

DNAbilize[®] Technology Summary

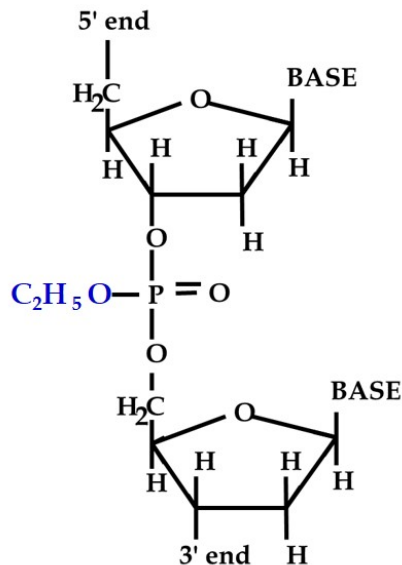
DNAbilize[®] Technology is a proprietary antisense RNAi nanoparticle platform that solves the antisense industry dilemma

SAFE: No toxicity from the DNA modification or the lipid nanoparticle delivery, no platelet or hepatic toxicity

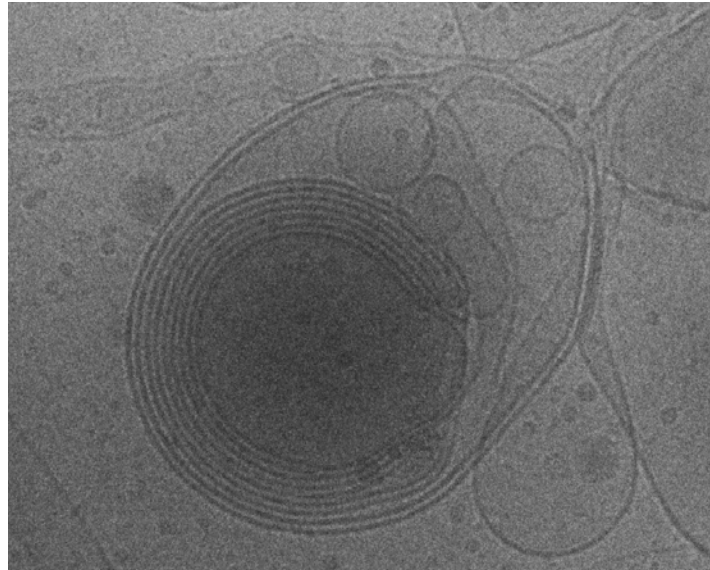
SYSTEMIC: Allows for systemic distribution via IV infusion

SPECIFIC: Knock down a single protein, no off-target effects observed

P-ethoxy backbone



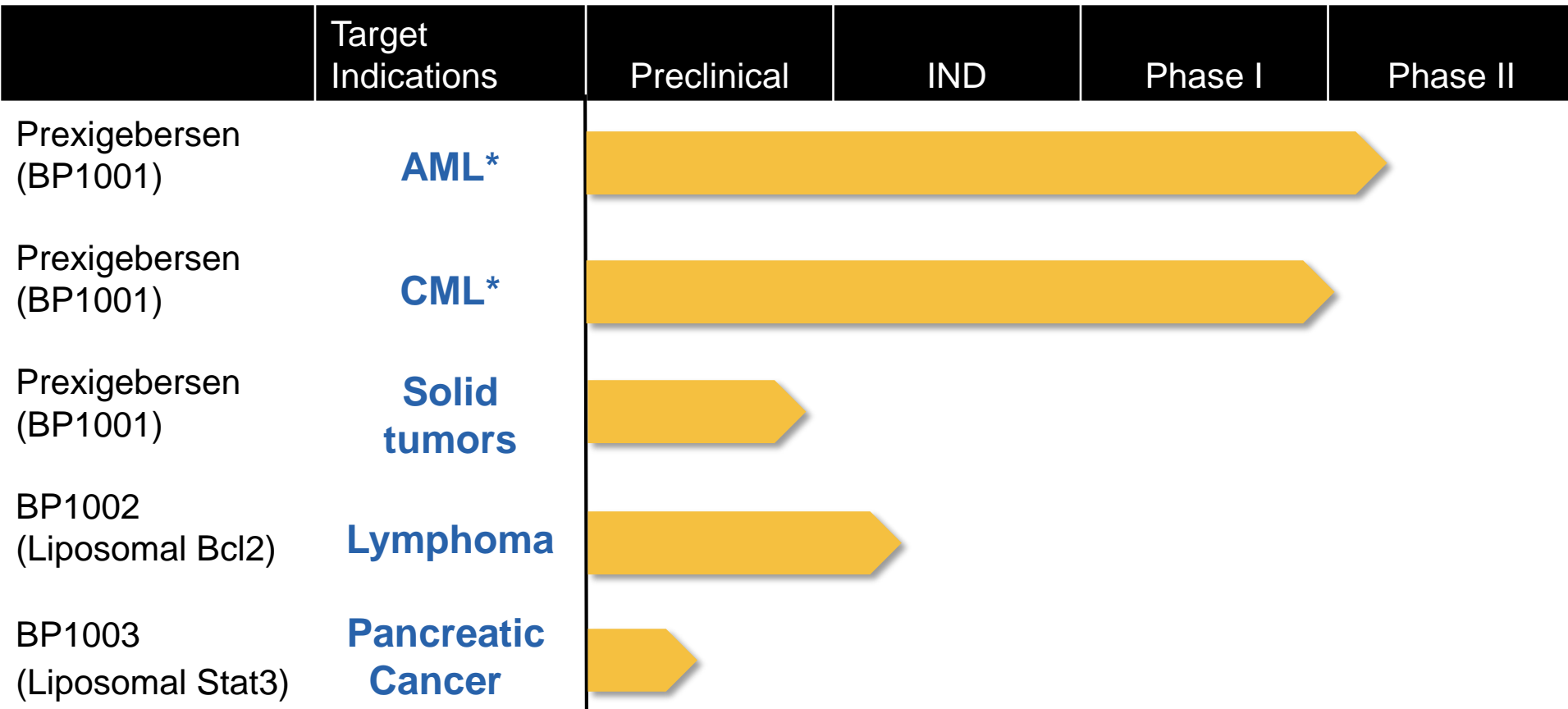
Neutral liposomes



DNAbilize[®]



Clinical Pipeline

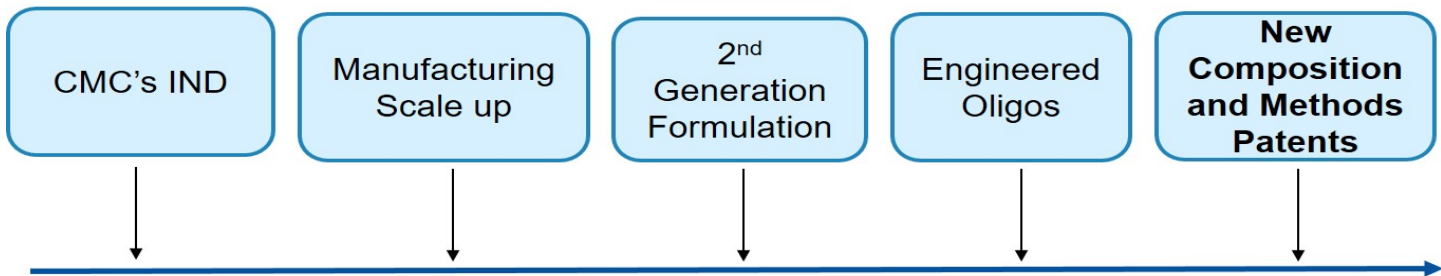


DNAbilize® Technology

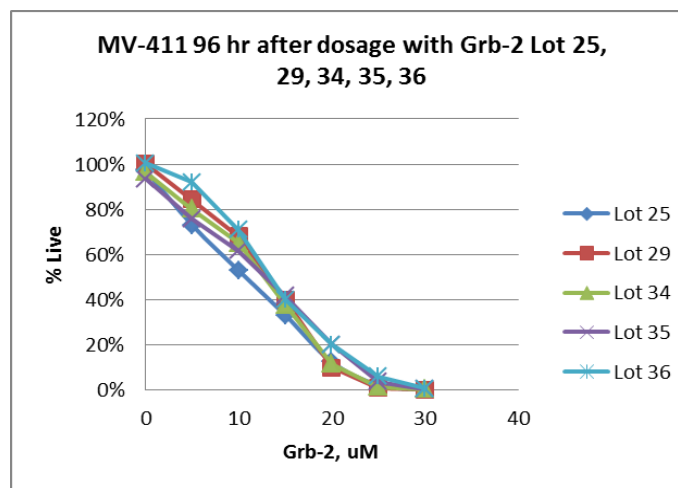
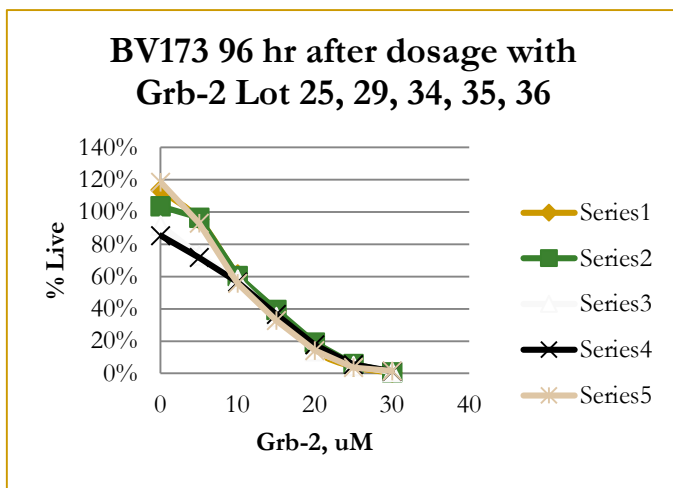
Ready to out-license

***Prexigebersen (BP1001) has received orphan drug designation from the U.S. FDA for AML and CML and from the European Medicines Agency (EMA) for AML**

Continuing Improvement in Product Design and Manufacturing



Several test batches have confirmed the potency of our currently manufactured drug product in AML cells

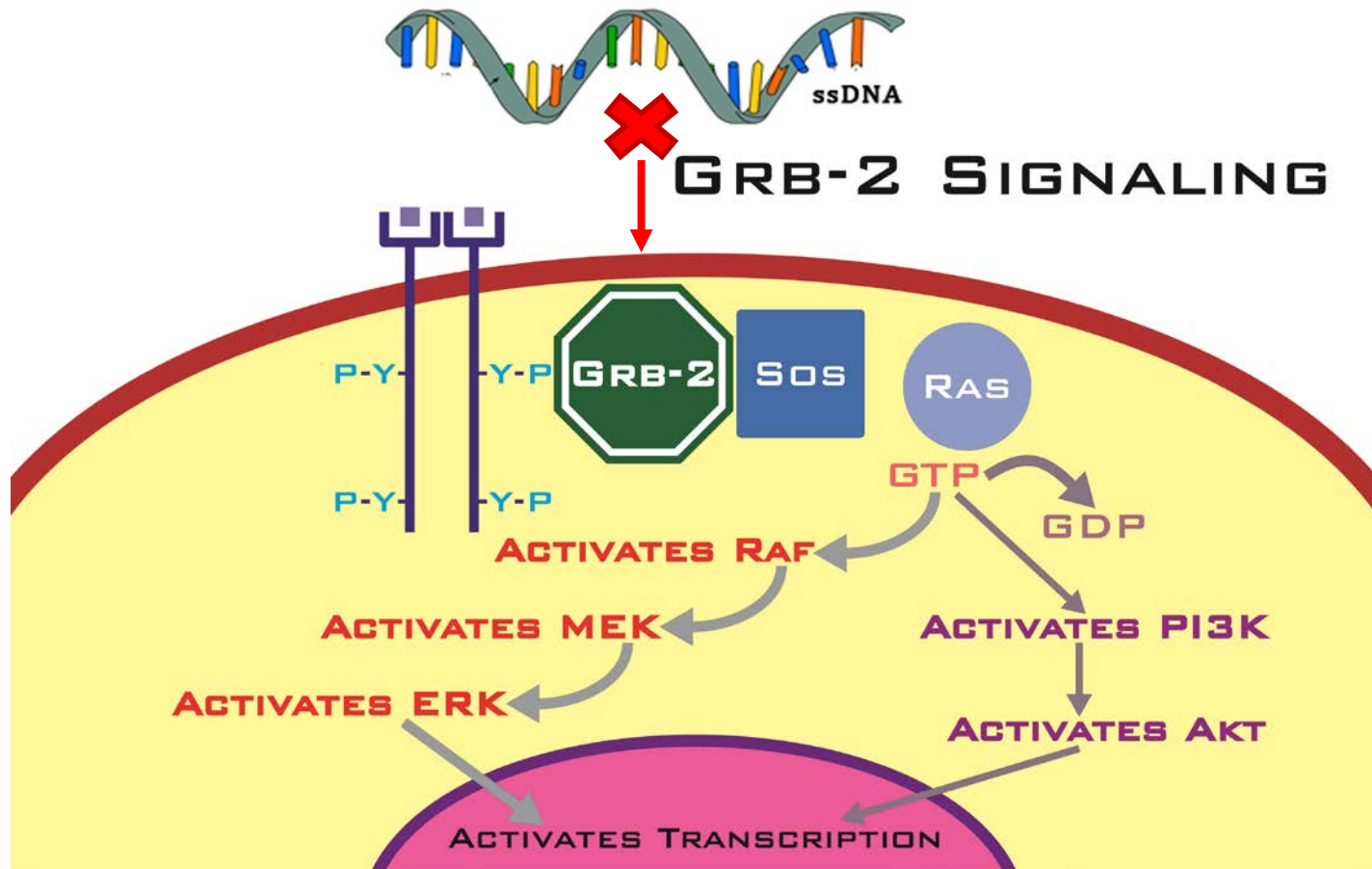


DNAbilize[®] Technology is rapidly being developed into a world class platform employing proprietary antisense RNAi nanoparticle technology

DNAbilize[®] Lead Target and Indications: Grb2 and Myeloid Leukemia

Prexigebersen is an antisense RNAi nanoparticle targeted to the protein Grb2

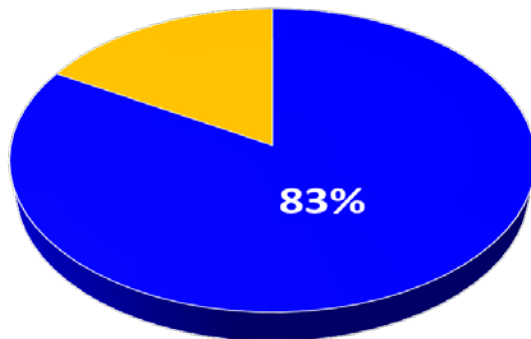
- ❖ Ideal target for myeloid leukemia because it shuts down the Ras pathway in receptor activated myeloid cells without exerting adverse effects on Ras signaling through other channels



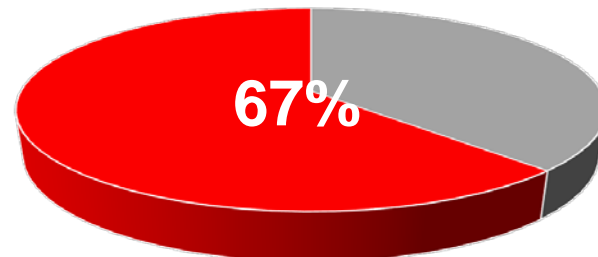
Summary of Phase I Monotherapy Clinical Trial Results for Prexigebersen

- AML, CML, & MDS Patients Refractory or Resistant to Current Therapies
- Patients averaged **6 prior therapies**
- Dose escalating treatment cycle, 8 doses over 4 weeks, up to 90 mg/m²
- Drug was well tolerated. One DLT (mucositis) occurred at the lowest dose (5 mg/m²) on the study in a patient with history of prior exposure to hydroxyurea which continued in study
- **Average reduction in circulating blasts was 67% in patient's showing a decrease in bone marrow blasts**
- Of the 18 evaluable patients with circulating blasts, **83% had a reduction in circulating blasts**

Evaluative patients blast count reduction



Reduction in circulating blasts in patients with a decrease in marrow blasts



Prexigebersen Down-regulates Grb2 Protein in Target Cells

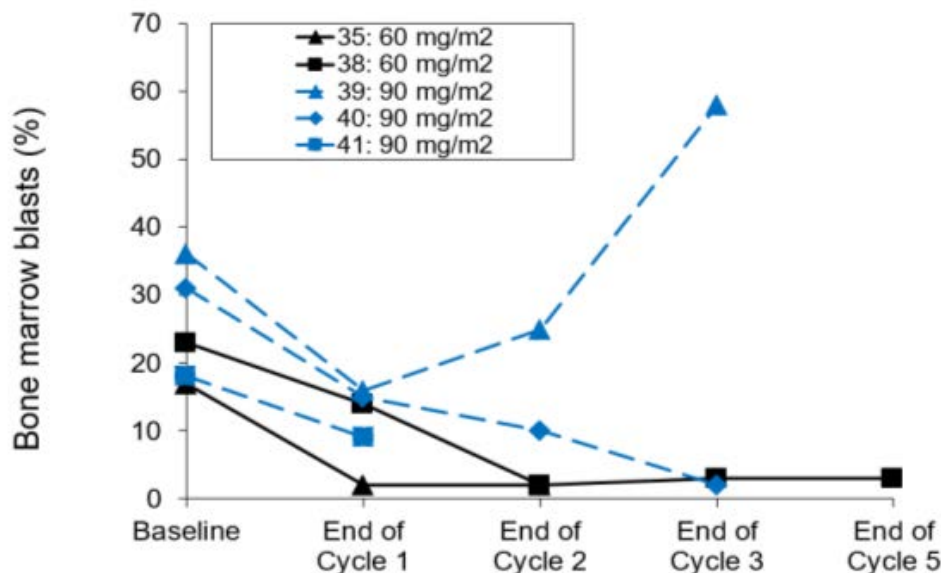
**Grb2 levels decreased in 11 of 13 patient samples by end of treatment (EOT)
pErk levels decreased in 7 of 13 patient samples by EOT**

Subject Number	Cohort	BP1001 dose (mg/m ²)	Grb2 Decrease (Day 15)	pErk Decrease (Day 15)	Grb2 Decrease (EOT)	pErk Decrease (EOT)
022	3	20	0%	0%	57%	0%
023	3	20	0%	3%	28%	45%
024	3	20	56%	28%	47%	35%
025	4	40	63%	82%	54%	91%
026	4	40	47%	0%	0%	0%
027	4	40	NS ¹	NS ¹	34%	27%
028	5	60	0%	0%	30%	54%
029	5	60	57%	51%	65% ²	0% ²
030	5	60	54%	55%	43%	47%
031	6	90	0%	0%	0%	0%
032	6	90	85%	54%	91%	63%
033	6	90	13%	13%	53%	2%
034	6	90	42%	42%	40%	0%

NS¹ = no sample collected

²Fewer cells used in analysis because sample had less cells

Safety Segment Phase II Prexigebersen + LDAC Combination Therapy Results



Relapsed/refractory AML patients

- 3 evaluable patients in each of 2 cohorts
- Dose 60 mg/m² and 90 mg/m² respectively
- Received drug 2x week for 4 weeks

5 of 6 patients responded:

- 3 patients achieved *complete remission*
- 2 patients achieved *partial remission*

Pharmacokinetics indicate prexigebersen has a 30 hr half life in plasma

No adverse effects attributed to prexigebersen were observed. MTD was not reached.

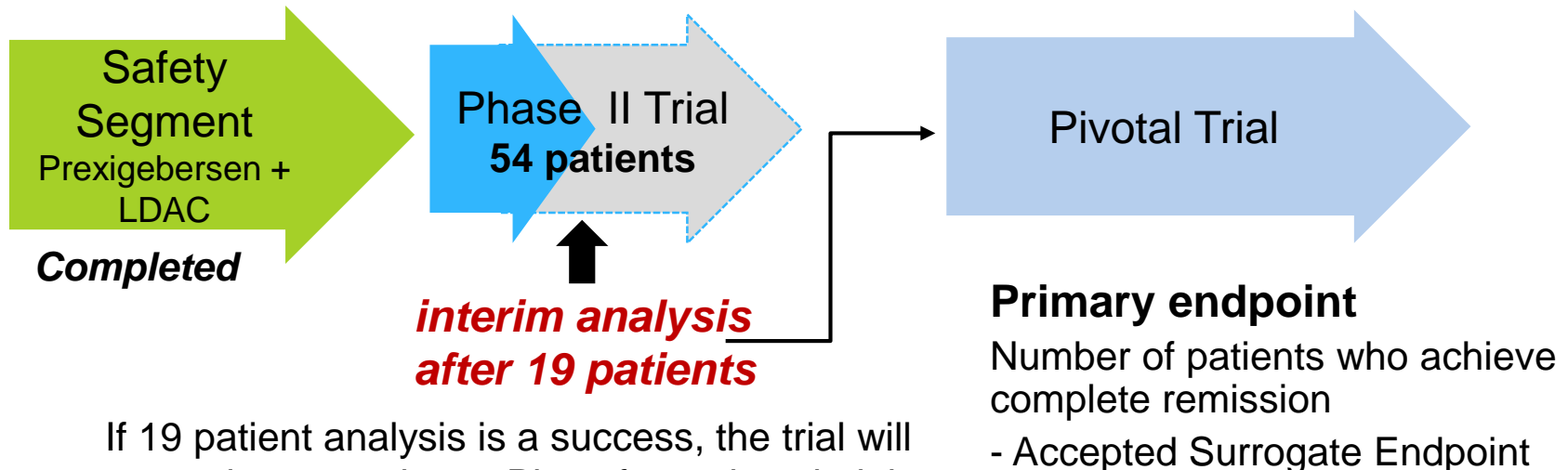
CR = Complete remission PR = Partial remission PD = Progressive disease

Patient	BP1001 (mg/m ²)	BM Blasts % Reduction	Cycles completed	Response
35	60	88	1	CR
37	60	0	1	PD
38	60	91	5	CR
39	90	56	3	PR
40	90	68	3	CR
41	90	50	3	PR

71% average reduction of bone marrow blasts in responding patients

Ongoing Phase II Efficacy Trial Design for AML Prexigebersen Combination Therapy

- Safety segment completed, demonstrated no negative synergies but significant efficacy using prexigebersen together with frontline therapy (LDAC) in refractory and resistant patients
 - Treatment of de novo AML patients who are induction therapy ineligible and unfit for a stem cell transplant
 - Efficacy trial will be conducted at up to 10 leading cancer centers in the U.S., including the MD Anderson Cancer Center; 6 sites have enrolled and treated patients



If 19 patient analysis is a success, the trial will expand to 54 patients. Plans for a pivotal trial will be discussed with FDA if results significantly exceed current therapy

Advances in the CML Program

Presentation at the December 2016 American Society of Hematology Annual Meeting

- *BP1001, a Novel Therapeutic for Chronic Myelogenous Leukemia*
- Prexigebersen (BP1001) decreased the proliferation of Gleevec® (imatinib)-resistant CML cells in a dose-dependent manner
- Prexigebersen pretreatment enhanced the inhibitory effects of Sprycel® (dasatinib) in CML cells, leading to cell death
- Two CML patients in Phase I with drug-resistant mutations showed significant reductions in circulating blasts during treatment, including one patient who went from 89% to 12% while another 24% to 7%

Clinical Program: Open for enrollment

- Phase II
 - Determine if there is a dose-limiting toxicity (DLT) of prexigebersen in combination with Sprycel®
 - Two cohorts of three evaluable patients at 60 mg/m² and 90 mg/m²
 - Compare the efficacy of prexigebersen in combination with Sprycel to historical response rates in blast phase and accelerated phase CML patients documented for Sprycel alone in 40 evaluable patients.

Prexigebersen has the potential to treat the 33% of CML patients who are resistant to Gleevec, the current standard of care

Prexigebersen Treatment Paradigm

- Frontline therapy for de novo AML patients who are induction therapy ineligible and unfit for a stem cell transplant
 - Prexigebersen combination with LDAC
 - Current response rate frontline therapy alone ranges 5% to 18% depending on patient condition
 - Prexigebersen to improve response rates significantly
 - Reduce treatment related mortality
- Salvage therapy for relapsed and refractory AML patients who have failed two prior regimens
 - Safety segment trial of prexigebersen combination with LDAC demonstrated complete or partial response in 5 of 6 elderly patients
 - Average patient age 75 years old
- Blast crisis and accelerated phase CML patients
 - Prexigebersen combination with frontline therapy
 - Current response rate frontline therapy alone is <30%
 - Average survival 9 months

Preclinical Pipeline

Validating DNAbilize® with Key Opinion Leaders:

- Establishing preclinical studies in triple negative and inflammatory breast cancer and advanced ovarian cancer with the MD Anderson Cancer Center
 - Advances in liposome properties enhancing solid tumor outcomes
- Developing clinical and preclinical targets in pancreatic cancer using a patient derived *ex vivo* tumor model developed by The MD Anderson Cancer Center
 - Delivery demonstrating the ability to penetrate stroma, the greatest inhibitor to effective pancreatic treatments
- Establishing DNAbilize® technology for systemic immunotherapy for glioblastoma in collaboration with Thomas Jefferson University
 - Antisense RNAi nanoparticle treatment demonstrating significant advantages versus non-delivery RNAi

Achievements and Upcoming Milestones

- ✓ Enrolling and treating patients in prexigebersen Phase II efficacy trial for AML
- ✓ Safety segment for the Phase II trial for blast and accelerated phase CML patients open for enrollment
- ✓ Completed BP1002 IND enabling studies
- ✓ New target BP1003 (DNAbilize® Stat3) initially being developed in pancreatic cancer
- ✓ Expanded solid tumor pre-clinical development of indications for prexigebersen including ovarian, triple negative and inflammatory breast cancer

Value propositions being advanced:

- 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
- Safety segment of the prexigebersen Phase II clinical trial for blast and accelerated crisis CML will provide insight into toxicity and potentially efficacy
- Demonstrating effectiveness of DNAbilize® antisense RNAi nanoparticle technology (broad drug development, licensing opportunities)
- Pursuing new manufacturing and target IP

Leadership

Peter Nielsen

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

- Officer and Director since founding Company in 2007
- Manufacturing development and evolution of engineered product design

William Hahne, M.D.

Vice President of Clinical Research

- Medical consultant for Medimmune, Lion Biotechnologies, Seattle Genetics, Aminex Therapeutics, Therakos, and Celgene Cellular Therapeutics
- Held executive positions in clinical research and medical affairs at Celator Pharmaceuticals, Celsion Corp, and CurGen Corp.

Ana M. Tari, PhD, MBA

Director, Preclinical Operations & Research

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

Anthony Price, MBA

Director, Finance & Accounting

- Former Associate Director of Accounting and Finance at Lexicon Pharmaceuticals

Suzanne Kennedy, PhD

Director, Corporate Development

- More than 17 years of marketing, business development, and research & development experience in the biotech industry

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

D. Craig Hooper, Ph.D.

- Professor of Cancer Biology and Neurological Surgery and the Section Chief for Translational Research, Tumor Division, in the Department of Neurological Surgery at Thomas Jefferson University
- His work has led to novel immune therapies for rabies infection, neuro-inflammation, and cancer with 10 issued U.S. patents and 19 international patents that have been licensed to five companies in the U.S. and abroad
- He is the founding president of the Jefferson Chapter of the National Academy of Inventors and a fellow of the National Academy of Inventors.

Recruiting additional members

IP and Financial Snapshot

Intellectual Property

- Original patents licensed from MD Anderson
- New composition and methods of use patent issued covers DNAbilize[®] technology, solely owned by Bio-Path
 - Five additional patents pending

Financial Snapshot

- **Ticker:** NASDAQ: BPTH
- **Cash:** \$4.6 million as of September 30, 2017
 - \$4 million registered direct offering in Q4 of 2017 provides sufficient cash to meet next key milestones
- **Market Cap:** Approximately \$25 million
- **Burn rate:**
 - Approximately \$1.5 million per quarter