



Company Overview October 2017

“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.

IP and Financial Snapshot

Intellectual Property

- Original patents licensed from MD Anderson
- New composition and methods of use patents filed to cover DNAbilize[®] technology, solely owned by Bio-Path extends protection for 20 years
 - Notice of allowance July 2017

Financial Snapshot

- **Ticker:** NASDAQ: BPTH
- **Cash:** \$6.1 million as of June 30, 2017
 - \$10 million registered direct offering and recent \$1.65 million warrant exercise extend cash runway through first half of 2018
- **Market Cap:** Approximately \$40-60 million
- **Burn rate:**
 - \$1 million per quarter core overhead
 - External programs: \$0.4-0.8 million per quarter

DNAbilize[®] Technology Platform

- ✓ Enables the development and delivery of systemic intravenous DNA treatments for a broad spectrum of cancers as well as diseases outside of cancer
- ✓ DNAbilize[®] is a **Platform Technology** with the potential to treat a number of oncology targets and other diseases
 - Only technology in therapeutic applications that has shown no evidence of toxicity while producing therapeutic effect
 - Ability to address hard to treat diseases and unmet clinical needs in fragile populations

Investment Highlights

Broad Vision, Focused Strategy

- Next generation DNA based therapeutics
- Original technology licensed from The MD Anderson Cancer Center with significant improvements from Bio-Path

Clinical Stage Pipeline

- Lead candidate, prexigebersen, in Phase II for acute myeloid leukemia and chronic myeloid leukemia
- Second drug candidate being readied to start Phase I

Novel Mechanism of Action

- Demonstrated ability to deliver DNA drug substance into target cells and down-regulate the target protein in systemic disease
- Lack of toxicity allows for development of drugs for hard to treat diseases and unmet needs among fragile populations, i.e. patients who cannot withstand frontline chemotherapy or radiation treatments

Robust Preclinical Pipeline

- Applications and development in other cancers including solid tumors and indications outside of cancer
- Prexigebersen is in preclinical development for treatment of solid tumors including ovarian cancer

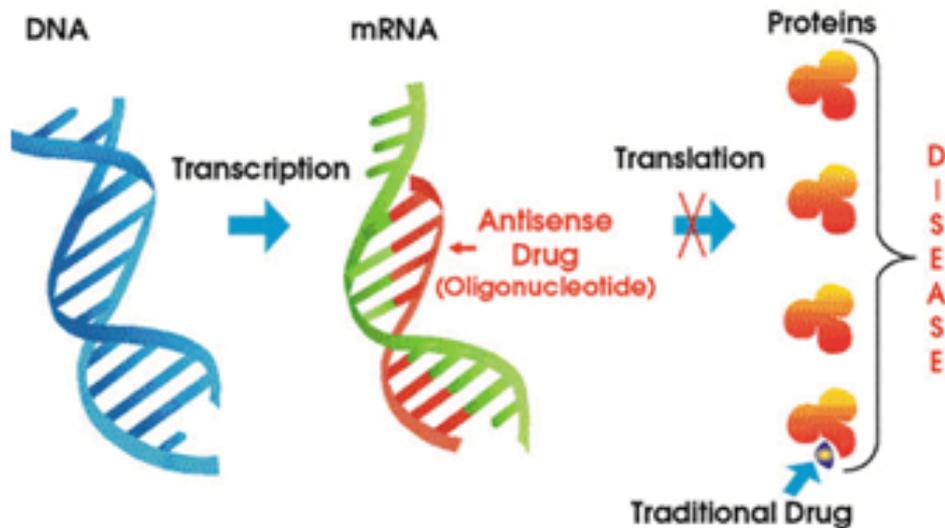
Validating Academic Collaborations

- UT Southwestern developing clinical and preclinical pipeline for lupus
- MD Anderson developing treatments for pancreatic, triple negative and inflammatory breast and advanced ovarian cancers
- Thomas Jefferson University establishing DNAbilize® Technology for brain cancer (glioblastoma)

DNAbilize® Technology Profile: 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 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
 - Does not induce bleeding side effects
- **Systemic treatment** - I.V. delivery to the diseased blood cells and main organs
- **Microscopic-sized liposomes** - enable penetration into diseased blood cells and tumors for delivery of drug
- **Proven target inhibition** - demonstrated that DNAbilize® method inhibits target protein, proving delivery technology works

Why Antisense DNA?



- Antisense DNA inhibits the production of *only* the protein of interest. The specificity of the treatment means less side effects for the patient
- Antisense DNA blocks the problem protein from being made. Other therapeutics attempt to inhibit a protein already in circulation. With antisense DNA, you stop the protein at its source
- Cancer resistance to antisense DNA therapeutics is less likely because the aberrant protein is shut off. Mutations to overcome the drug or recruitment of alternative proteins is hampered when the protein is shut off.

Antisense DNA is stable, easy to design, and easy to manufacture, evaluate and validate in cells.

DNAbilize[®] antisense delivery system is the fast track to the clinic

DNAbilize® Technology Summary

Core of the technology is a combination of a stable DNA backbone and a neutral lipid (fat cell) delivery that mimics the cell membrane

SAFE: No toxicity from the DNA or the lipid delivery, no blood clotting or liver toxicity

SYSTEMIC: Allows for whole body distribution via IV infusion

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

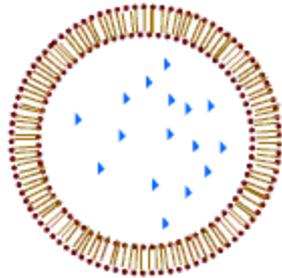
DNAbilize®



Liposomal Drug Uptake by Cells

Inhibitory DNA incorporated into liposomes that resemble the cell membrane seamlessly merge with the cell, releasing the DNA drug into the cytoplasm

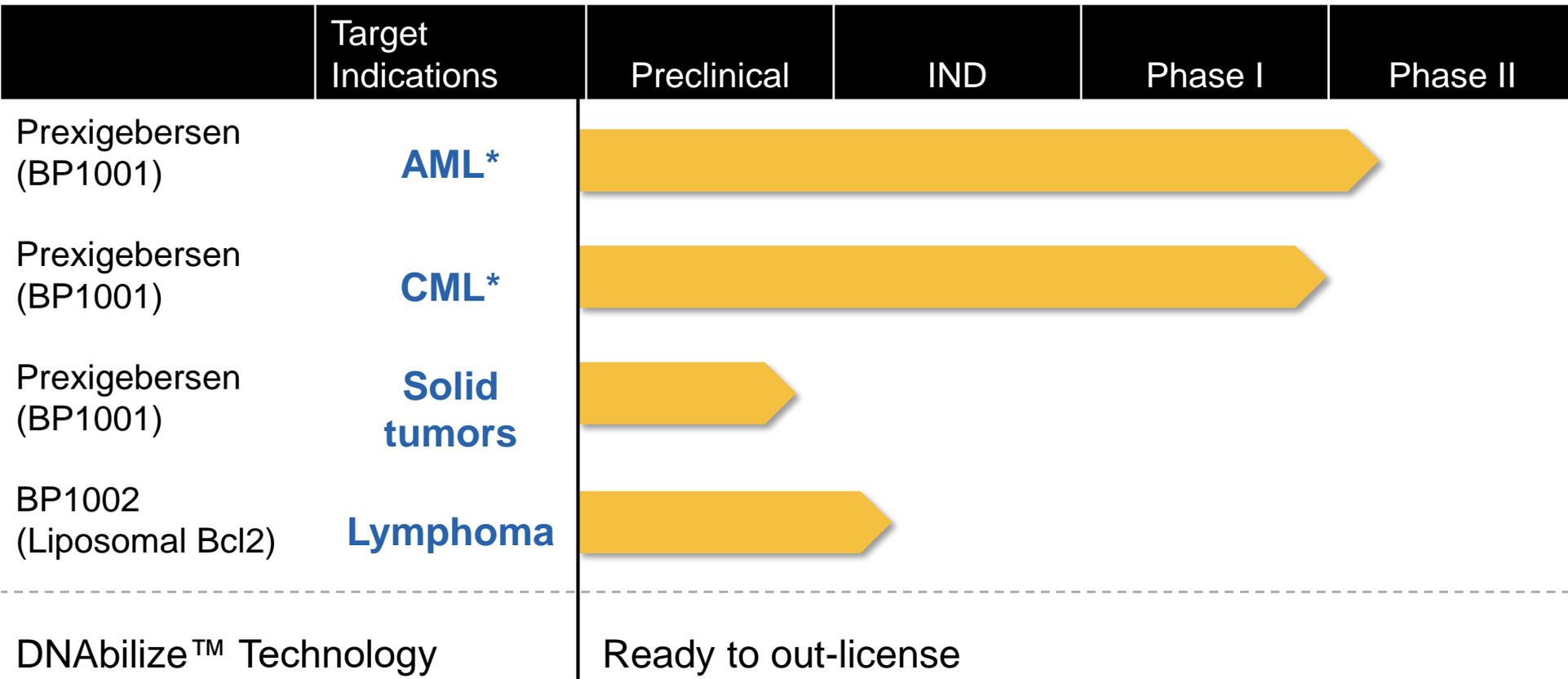
Outside the diseased cell:
Liposome in the blood stream
carrying DNAbilize technology
drug product



Inside the diseased cell:
Significant uptake of the
DNAbilize technology to
treat targeted disease



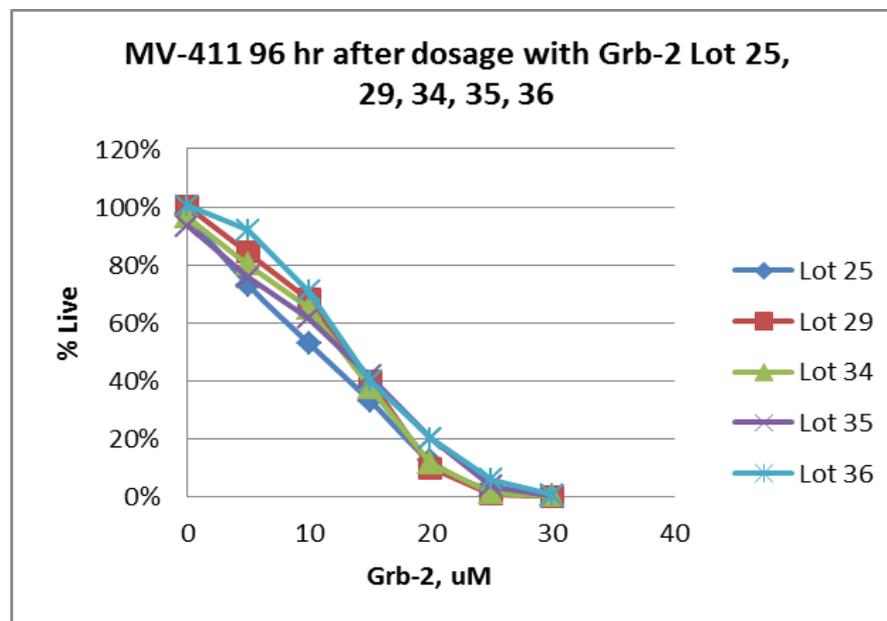
Clinical Pipeline



****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 Improvements in Product Manufacturing and Design

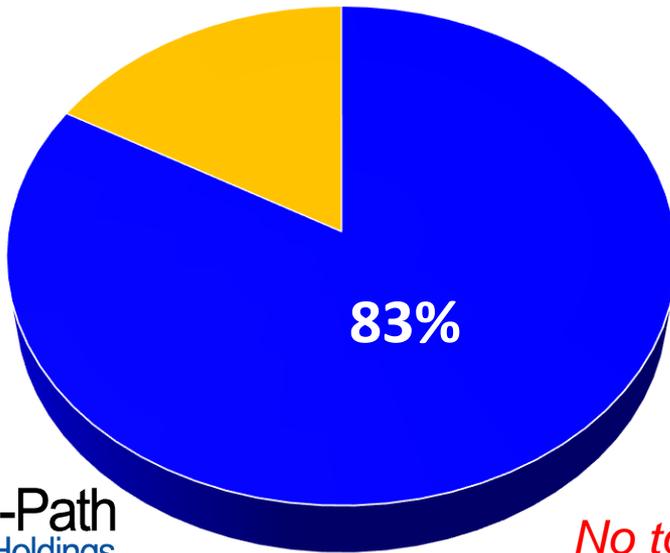
- Through our dose-escalating Phase 1 trial we upgraded our supplier base with the goal of increased capability and capacity
- Several test batches have confirmed the potency of our currently manufactured drug product in AML cells.
- Identical and reproducible efficacy results from five different lots of manufactured product demonstrates excellence in manufacturing



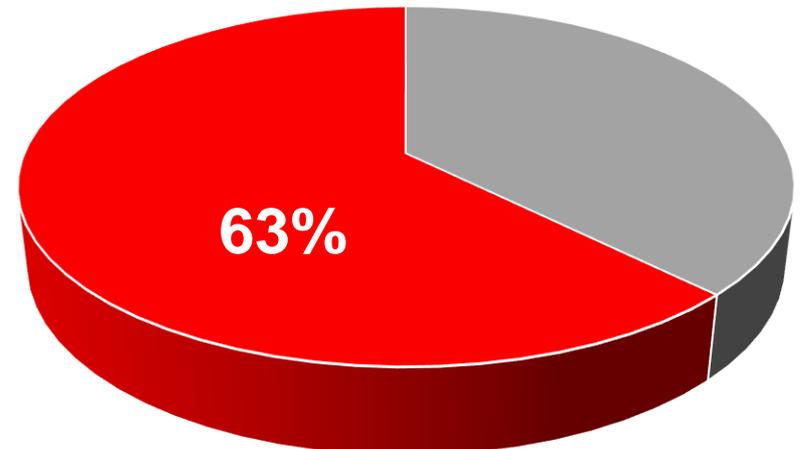
Summary of Phase I Monotherapy Clinical Trial Results for Prexigebersen

- Target of drug is a protein called Grb2, important in growth and proliferation of cancer cells
- Phase I trial for 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
- **Average reduction in circulating blasts (leukemic cells) was 67% in patient's showing anti-leukemic response to drug**
- Of the 18 evaluable patients with circulating blasts, **83% had a response to the drug**

Evaluable patients responding to drug



Evaluable patients with >50% reduction in circulating blasts



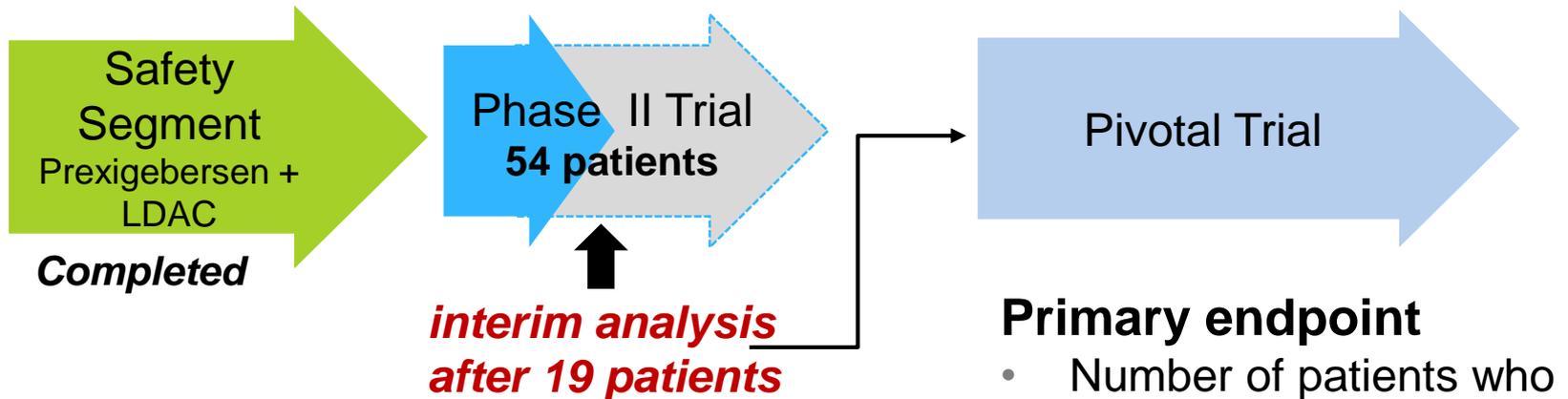
No toxic side effects!

Safety Segment Phase II Prexigebersen + LDAC Combination Therapy Results

- Low Dose Ara C (LDAC) is the current frontline treatment for elderly AML
- The safety trial for the combination of LDAC + prexigebersen was evaluated in relapsed AML patients, average age 75 years old
 - 3 evaluable patients in each of 2 cohorts
 - The two highest doses evaluated; 60 mg/m² and 90 mg/m² respectively
 - Patients received drug twice a week for 4 weeks
- **No serious adverse effects attributed to prexigebersen were observed. The maximum tolerated dose was not reached**
- 5 of 6 patients responded; 3 patients achieved **complete remission** and 2 patients achieved **partial remission**

Ongoing Phase II Efficacy Trial Design for AML Prexigebersen Combination Therapy

- Safety segment completed, demonstrated no negative synergies using prexigebersen together with frontline therapy, low dose chemotherapy (LDAC)
 - Treatment of newly diagnosed AML patients who are fragile
 - Efficacy trial will be conducted at 10 leading cancer centers in the U.S., including the MD Anderson Cancer Center; 6 sites are enrolling patients now



If interim analysis successful, the trial is expected to be rolled into a **pivotal trial** for accelerated approval

Primary endpoint

- Number of patients who achieve complete remission

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

Preclinical Pipeline

Validating DNAbilize™ with Key Opinion Leaders:

- Establishing prexigebersen 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 for treatment of systemic lupus in collaboration with UT Southwestern Medical Center
 - Stabilizing immune system properties
- 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
 - DNA nanoparticle delivery demonstrating significant advantages

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
- ✓ Received orphan drug designation in the EU from the European Medicines Agency for AML
- ✓ Expanded pre-clinical development with new target drug candidates (lymphoma, pancreatic, brain, autoimmune disease)
- ✓ 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
- Estimated completion of the interim analysis by end of 2017
- 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

Tara Sadeghi, MPH

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 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

Competitors with DNA/RNA Therapeutics

<u>Company</u>	<u>Bio-Path</u>	<u>Ionis</u>	<u>Anylam</u>	<u>Dicerna</u>
<u>Drug</u>	DNA	DNA	siRNA	siRNA
<u>Delivery</u>	Uncharged lipids	None	Sugar	Sugar
<u>Safety</u>	Non-toxic	Blood clotting toxicity	Liver toxicity	Liver toxicity
<u>Symbol</u>	BPTH	IONS	ALNY	DRNA
<u>Share price</u>	\$0.41	\$52.93	\$121.07	\$6.27
<u>Market cap</u>	\$50 M	\$6.5 B	\$11.1 B	\$130.4 M