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Partnering overview

Q4 2020

BridgeBio's mission is to accelerate therapies for genetic diseases

Our Core Values

1. **Put patients first** – Bridge the gaps between scientific possibility, business case, and treatments for patients
2. **Think independently** – "First principles" mindset; unafraid to question assumptions
3. **Be radically transparent** – A culture of open communication where every perspective is heard
4. **Every minute counts** – Deliver treatments from discovery to patients as fast as humanly possible
5. **Let science speak** – Every potential medicine is judged by its scientific merits first

We aspire to partner with leading research organizations to translate genetically-defined therapies

Our Partnership Philosophy

1. **Decisions are patient-centric** – We only support research if we believe it is the best possible decision for patients, and we are the best possible partner
2. **Marathon, not a sprint** – The best relationships are built over the long-term. We are not here just to license IP, but to facilitate a feed forward loop from idea to drug
3. **Risk and reward are always shared** – NewCo creation from licensed products will reward everyone if successful

Typical alliance structure

- 3-year duration
- Annual engagement with PIs directly through Zoom or RFP
- Possibility to sponsor research or license and create NewCo
- Detailed feedback for every opportunity we come across

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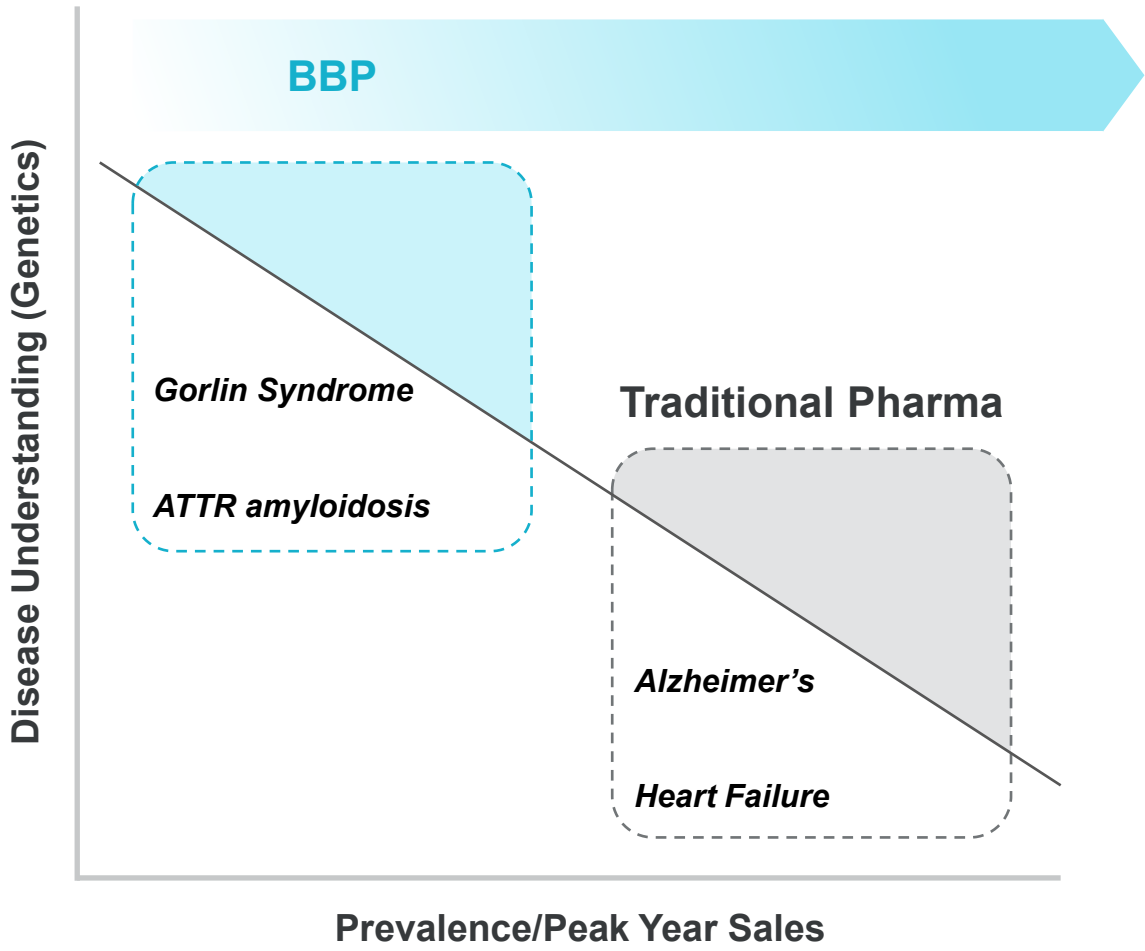
Agenda

How we work

How we partner

Portfolio snapshots

BridgeBio Pharma: A new type of company, designed for the opportunity in genetic disease



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- Target diseases at their source
- World-class R&D
- Hyper-efficient execution
- Structural focus at the level of each asset/de-centralized
- Opportunistic by modality, stage, therapeutic area

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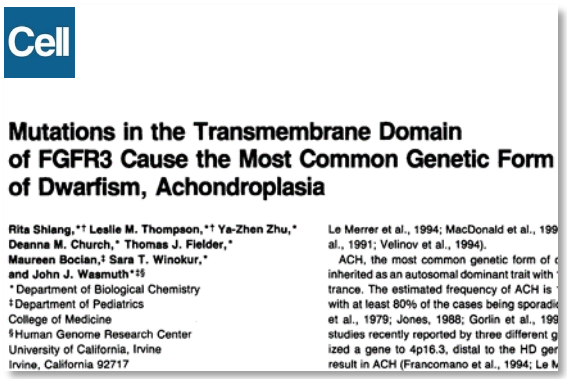
We exist to leverage genetic findings into new therapies

1

Characterize the genetic source of the disease



Cell

Mutations in the Transmembrane Domain of FGFR3 Cause the Most Common Genetic Form of Dwarfism, Achondroplasia

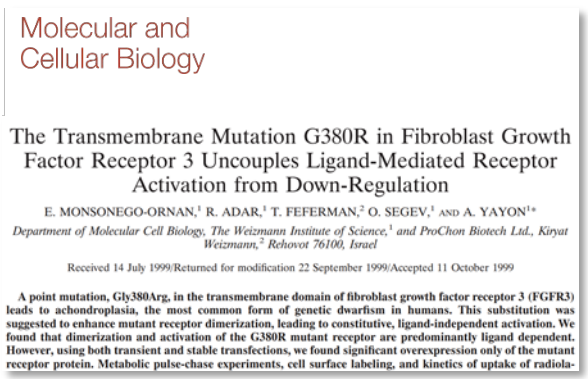
Rita Shiang,*† Leslie M. Thompson,*† Ya-Zhen Zhu,* Deanna M. Church,* Thomas J. Fielder,* Maureen Bocian,‡ Sara T. Winokur,* and John J. Wasmuth*‡§
*Department of Biological Chemistry
‡Department of Pediatrics
College of Medicine
§Human Genome Research Center
University of California, Irvine
Irvine, California 92717

Le Merrer et al., 1994; MacDonald et al., 199 al., 1991; Velinov et al., 1994).

ACH, the most common genetic form of c inherited as an autosomal dominant trait with trance. The estimated frequency of ACH is with at least 80% of the cases being sporadi et al., 1979; Jones, 1988; Gorlin et al., 199 studies recently reported by three different g ized a gene to 4p16.3, distal to the HD ger result in ACH (Francomano et al., 1994; Le M

2

Define the mechanism of the genetic drivers



Molecular and Cellular Biology

The Transmembrane Mutation G380R in Fibroblast Growth Factor Receptor 3 Uncouples Ligand-Mediated Receptor Activation from Down-Regulation

E. MONSONEGO-ORNAN,[1] R. ADAR,[1] T. FEFERMAN,[2] O. SEGEV,[1] AND A. YAYON[1]*
Department of Molecular Cell Biology, The Weizmann Institute of Science,[1] and ProChon Biotech Ltd., Kiryat Weizmann,[2] Rehovot 76100, Israel

Received 14 July 1999/Returned for modification 22 September 1999/Accepted 11 October 1999

A point mutation, Gly380Arg, in the transmembrane domain of fibroblast growth factor receptor 3 (FGFR3) leads to achondroplasia, the most common form of genetic dwarfism in humans. This substitution was suggested to enhance mutant receptor dimerization, leading to constitutive, ligand-independent activation. We found that dimerization and activation of the G380R mutant receptor are predominantly ligand dependent. However, using both transient and stable transfections, we found significant overexpression only of the mutant receptor protein. Metabolic pulse-chase experiments, cell surface labeling, and kinetics of uptake of radiola-

3

Drug the molecular pathophysiology at its source

news release

bridgebio pharma's qed therapeutics doses first child in phase 2 clinical trial of the investigational medicine infigratinib in achondroplasia

15.07.2020 at 7:30 AM EDT

SAN FRANCISCO, July 15, 2020 (GLOBE NEWSWIRE) -- BridgeBio Pharma, Inc. (Nasdaq: BBIO) affiliate QED Therapeutics announced today that the first child with achondroplasia has been dosed with the investigational medicine infigratinib, an orally available small molecule, that targets the overactivity of fibroblast growth factor receptor 3 (FGFR3) in the PROPEL 2 Phase 2 clinical trial. Achondroplasia is the most common cause of disproportionate short stature.[1]

"With preclinical evidence showing increased growth in the long bones, spine and cranial bones, including the base of the skull, there is the potential for infigratinib to help children with achondroplasia by decreasing the impact of serious medical complications," said Professor Ravi Savarirayan, M.B., B.S., M.D., Ph.D., clinical geneticist and group leader of skeletal biology and disease at Murdoch Children's Research Institute in Australia and the lead investigator for the PROPEL 2 trial. "Additionally, infigratinib is being studied as a once-daily dose taken orally, which is an important factor for administration of therapies to children with achondroplasia."

When the genetic driver of a disease is known, patients can be molecularly-matched to therapy

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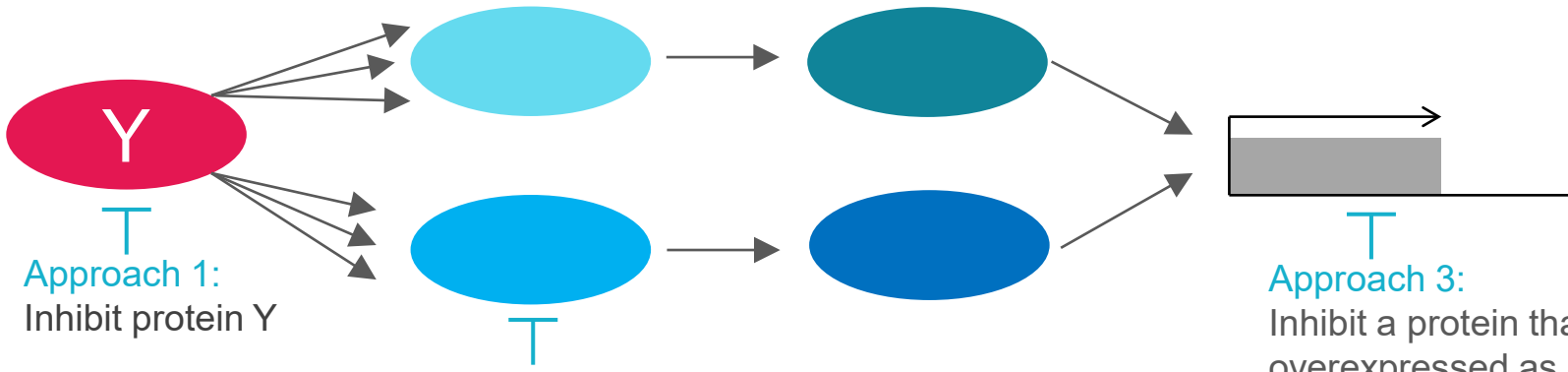
Drugging the source of pathology is key to our model

Hypothetical mechanism of Disease X

- Disease X is caused by increased signaling in protein Y due to a mutation
- Protein Y signaling is through two routes, causing gene expression

Cartoon of Disease X signaling with possible therapeutic approaches



Interpretation of therapeutic approaches

- Approach 1: The disease is being drugged at the source. Even better if the drug is mutant-specific.
- Approach 2: The disease is being drugged one degree away from the source. Not all pathways affected by the mutant are addressed by the therapy
- Approach 3: The disease is being drugged several degrees away from the source.

We are led by industry veterans

We rely on some of the top R&D minds in this industry to select new therapies…

Charles Homcy, MD
Chairman of Pharmaceuticals

PORTOLA PHARMACEUTICALS · MyoKardia · GBT · Takeda MILLENNIUM THE TAKEDA ONCOLOGY COMPANY

VELCADE (bortezomib) FOR INJECTION · Andexxa Coagulation Factor Xa (Recombinant), inactivated - zhzo · Oxbryta (voxelotor) 500 mg tablets

Frank McCormick, PhD, FRS
Chairman of Oncology

Celtus · ONYX PHARMACEUTICALS · UCSF

Nexavar (sorafenib) tablets · Kyprolis (carfilzomib) for injection

Richard Scheller, PhD
Chairman of R&D

Genentech · 23andMe

TECENTRIQ atezolizumab · PERJETA pertuzumab for injection · Kadcyla trastuzumab emtansine · OCREVUS ocrelizumab · VENCLEXTA venetoclax tablets

…and put them in the hands of seasoned industry R&D operations

Uma Sinha, PhD
Chief Scientific Officer

GBT · PORTOLA PHARMACEUTICALS · Takeda MILLENNIUM THE TAKEDA ONCOLOGY COMPANY

(eptifibatide) Injection INTEGRILIN · Andexxa Coagulation Factor Xa (Recombinant), inactivated - zhzo · Oxbryta (voxelotor) 500 mg tablets

Eli Wallace, PhD
CSO in Residence, Oncology

ARRAY BIOPHARMA · Peloton Therapeutics

MEKTOVI (binimetinib) 15 mg tablets · Tucatinib (HER2i, Ph3) · PT2997 (HIF2αi, Ph3)

Robert Zamboni, PhD
Chemistry

MERCK FROSST

SINGULAIR (montelukast, MSD) · ARCOXIA (etoricoxib) · VIOXX (rofecoxib, MSD)

Together, our R&D team is responsible for 100+ INDs and 20+ approved products

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Our team is built to rapidly seek and develop new drugs

Area of expertise	Description	
Genetic disease	▪ Former or current academic leaders in medical genetics and genetic cancer research ▪ Strong network with hospital and academic Centers of Excellence	✓
Preclinical development	▪ Biologists, toxicologists, CMC, PK experts, and other R&D specialties ▪ Medicinal chemists capable of optimizing small molecule drugs ▪ Strong track record advancing drugs into the clinic	✓
Clinical and regulatory	▪ Experts in clinical trial design and execution, especially in orphan diseases ▪ Strong track record working with regulatory agencies to advance drugs to approval	✓
Program diligence	▪ Source new translational research and lead diligence for new investments ▪ Develop scientific and business cases for new company creation	✓
Novel modalities	▪ Experience designing oligonucleotide, AAV, and protein-based therapies ▪ Understanding of modality-specific preclinical and clinical development	✓
Operations	▪ Owner of each portfolio company's development plan, business strategy, budget	✓

We have a uniquely dedicated and experienced team focused on advancing our current genetic disease programs, as well as sourcing new program opportunities

Together we are advancing >20 diverse therapeutics

Small molecule | Topical small molecule | Biologics | Gene therapy

= Snapshots of this program in the last section

Portfolio segment	Program[1]	Drug mechanism	Diseases	Patient pop. (US+EU)	Modality	Pre-Clinical: Discovery	Pre-Clinical: IND-enabling	Clinical: Phase1	Clinical: Phase 2	Clinical: Phase 3
Mendelian	**AG10**	TTR stabilizer	ATTR-CM	>400K						
	BBP-870	cPMP replacement	MoCD type A	100						NDA
	Infigratinib	Low-dose FGFR1-3i	Achondroplasia[3]	55K						
	Encaleret	CaSR antagonist	ADH1 / HP	12K / 200K						
	Zuretinol	Synthetic retinoid	IRD (RPE65 or LRAT)	3K						
	BBP-418	Glycosylation substrate	LGMD2i	7K						
	BBP-711	GO1 inhibitor	PH1 / FSF	5K / 1.5M						
	BBP-671	PanK activator	PKAN / OA	7K						
	BBP-761	Succinate prodrug	LHON	20K						
	BBP-472	PI3Kβi	PTEN autism	120K						
Genetic Dermatology	**Patidegib[2]**	Topical SMOi	Gorlin / BCC	120K						
	BBP-589	Recombinant COL7	RDEB	1.5K						
	BBP-681	Topical PI3Kαi	VM / LM	117K						
	BBP-561	Topical KLK 5/7i	Netherton	11K						
Targeted Oncology	**Infigratinib**	FGFR1-3i	FGFR+ tumors	37K						
	BBP-398	SHP2i	Multiple tumors	>500K						
	BBP-454	Pan-mutant KRASi	KRAS+ tumors	>500K						
	BBP-954	GPX4i	Multiple tumors	>500K						
Gene Therapy	**BBP-631**	21-OH gene therapy	CAH	>75K						
	BBP-812	ASPA gene therapy	Canavan	1K						
	BBP-815	TMC1 gene therapy	Genetic hearing loss	10K						

[1] Each of our programs is housed in a separate affiliate company

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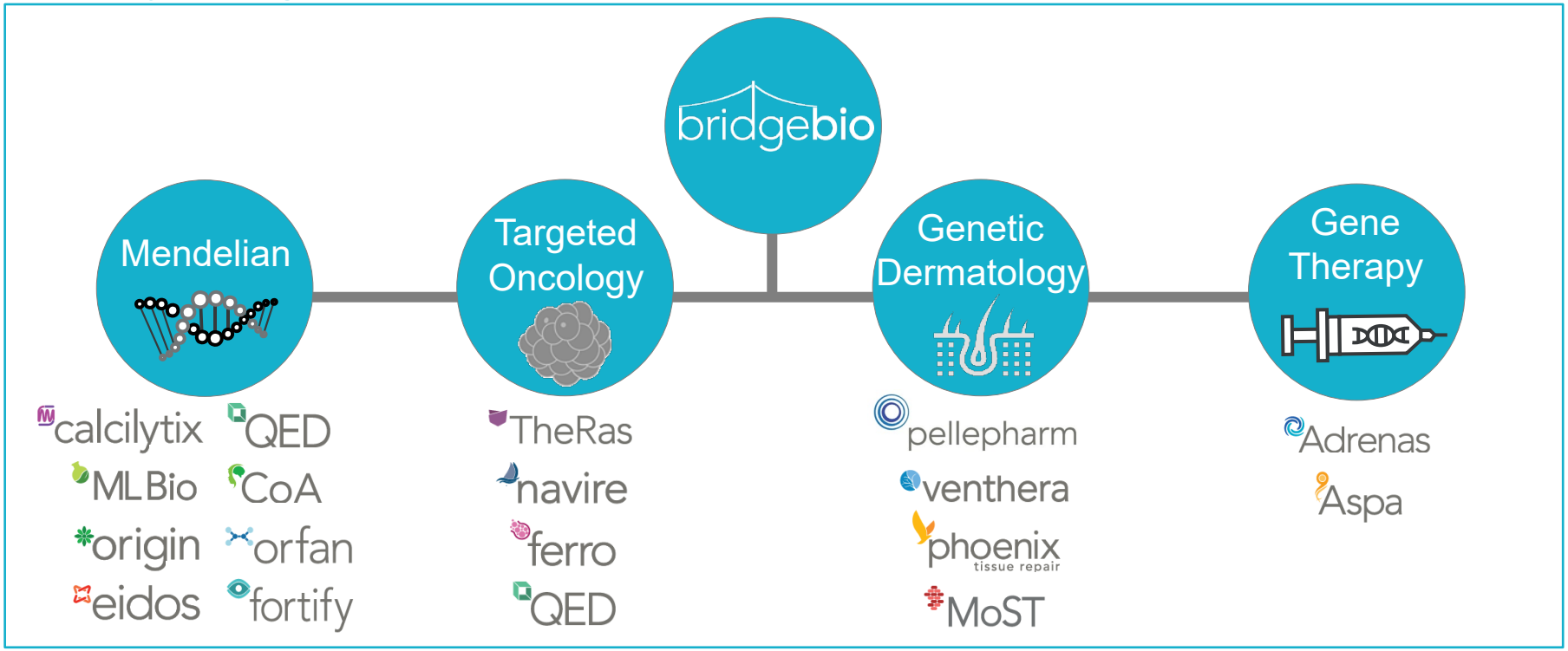
Each therapeutic has its own team and decision makers

The family of BridgeBio subsidiaries



Why we believe every therapy deserves to be separately housed

- Having a dedicated team for each subsidiary means each therapy is treated like a “lead therapy”, and not just another pipeline project
- Employee incentivization is aligned with the success of their drug, and ultimately with delivering a therapy to patients
- There are more options to advance or partner each therapy

Agenda

How we work

How we partner

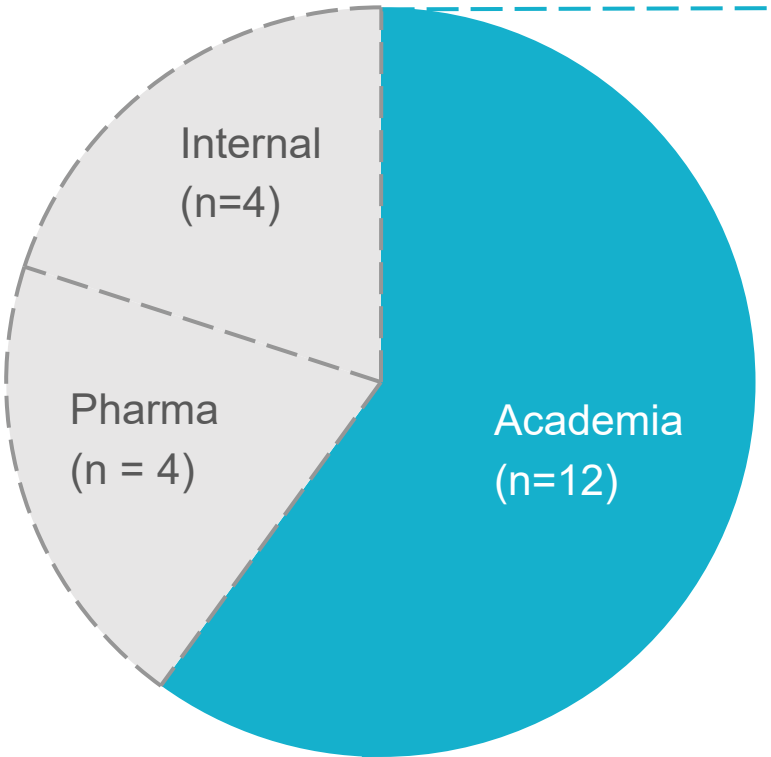
Portfolio snapshots

To increase our transparency to partners, we have established an FAQ about how we find and fund therapies

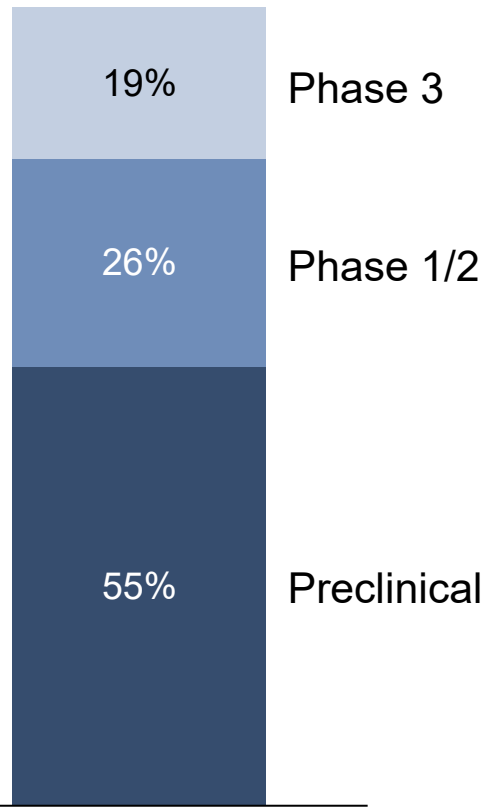
1. From where do our therapies originate?
2. What funding mechanisms exist?
3. How do we engage TTOs and PIs to learn about new research?
4. Who have we partnered with so far?
5. What are the properties of translational research that matter to us?
6. What kind of diligence do we do “behind the scenes”?
7. What does a successful investment look like over time?
8. What if the idea is on target, but the technology is too early?

Most of our investments originate from academia

Type of BridgeBio program

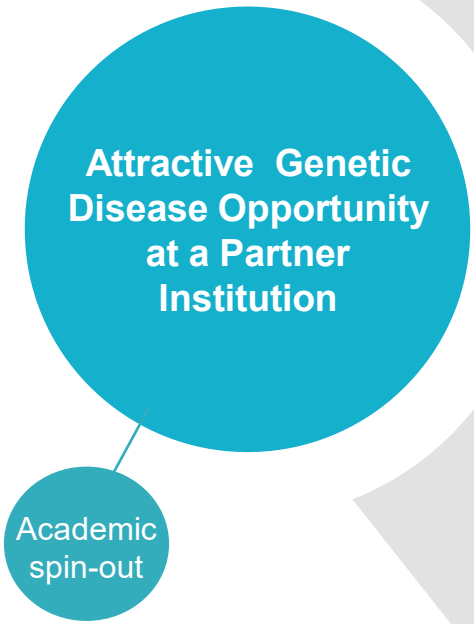


Current stage of programs brought in from academia



- We partner with academia to drive our engine of new programs, and we always seek to maintain a high degree of academic investments

There are 3 ways we fund translational science



1. Provide funding for a specific project

- BridgeBio sponsors research (SRA)
- For projects that still need a few key experiments
- Designed to lead into NewCo after 1-2 years

2. Start a new start-up

- BridgeBio supplies an equity investment and team
- For projects with preclinical "proof of concept" data, and IP
- NewCo can be based anywhere, doesn't need to be in CA

3. Invest in existing start-up

- BridgeBio supplies an equity investment
- For academic spin-outs that fit our investment scope

- The goal is always to make the business choice that objectively will deliver a better drug to patients as soon as possible
- If you are interested in speaking to us about partnering, please contact Eric Gomez at eg@bridgebio.com

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We prefer to cultivate long-term relationships with PIs

Outreach	Description	Pros	Cons
Ad hoc check-ins	▪ Sync with TTOs at partnering events ▪ Periodically connect about available technologies and startups	▪ Maintains point of contact	▪ Doesn't build long-term engagement ▪ Possibly misses nascent programs ▪ Less feedback from us
Scheduled "focus sessions" with PIs	▪ Curate research projects for hour-long discussions with PIs ▪ Our team studies each opportunity beforehand	▪ Relationship building ▪ PI gets real-time feedback and questions	▪ Research projects may be early ▪ PI may not be as familiar with us beforehand
Institution-wide request for proposal (RFP)	▪ Integrate into existing institution RFPs ▪ Or roll out a BridgeBio-specific RFP	▪ PIs often are more comfortable writing about their research ▪ The proposal scope can be narrowly crafted	▪ PIs may have proposal or grant-writing fatigue ▪ Slower timeline
Multi-year partnership	▪ A commitment to conduct RFPs for multiple consecutive years	▪ Can be adapted to include other activities like educational seminars or seats on advisory panels	▪ Requires effort from both sides to agree on legal terminology

Regardless of outreach type, we always try to give constructive feedback and educate investigators about our interests and how they can best position their research for successful translation

4. Who have we partnered with so far?

We are collaborating with 10+ universities currently

- We have executed 10+ multi-year alliances with universities (publicly disclosed ones listed below)
- We have held 10+ focus sessions with universities

Publicly disclosed academic/research institution collaborations

Johns Hopkins University

Boston Children's
Until every child is well™

Stanford University

Cincinnati Children's™

UCSF Health

The University of Texas MD Anderson Cancer Center

UF University of Florida

University of Colorado Anschutz Medical Campus

St. Jude Children's® Research Hospital

Our decision to fund is primarily based on 4 criteria

Genetics

- Is the disease characterized by a mutation?
- Is the therapy targeting the mutated protein or directly addressing the mutation's effect?
- Is there evidence the mutation is driving the pathology?

Molecular Mechanism

- How does the mutation change cell signaling?
- How does the mutation affect the structure-activity relationship of the target?
- Are there reliable animal models of the disease?

Patient Journey

- Is there a genotype-phenotype correlation?
- Is there natural history data?
- What do patients & families want addressed?
- What about the current standard of care are we improving?

Product

- Is there IP associated with the therapy?
- Is this a proven modality? (ex. antibody, AAV)
- How many patients will this product help?

Other considerations:
- We do not invest in platforms (ex. delivery vehicles), or cell therapies
- We invest in any disease area

If a proposal meets most, but not all criteria, we may invest through our incubator program to fund "gap" experiments

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It is important to be able to “connect the dots”

Example of connecting the dots for genetic diseases caused by mutations in GNAS

MUTATION

- GNAS mutations at R201C/H cause fibrous dysplasia and McCune-Albright Syndrome

PROTEIN DYSREGULATION

- R201C/H are gain-of-function variants that keep Gαs active regardless of GDP/GTP state, enhancing binding to adenylyl cyclase

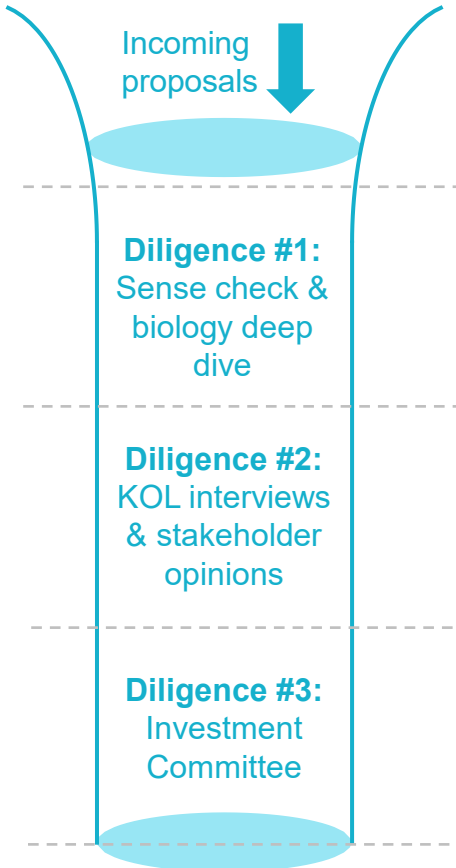
SIGNALING DYSREGULATION

- Binding to adenylyl cyclase increases cAMP levels, activating PKA. PKA activates transcription factors known to cause proliferation and growth

SYMPTOMS

- McCune-Albright is caused by a mosaic pattern of upregulated GNAS signaling in bone stromal stem cells and hormonal tissues
- Main symptoms include café-au-lait spots, endocrinopathies, and fibrous dysplasia of bone

Each opportunity is subject to 3 phases of diligence



Examples of questions we ask

- Have we talked to this institution or PI before?
- How can we increase our exposure to new potential partners?
- Does this meet our four main criteria?
- Does this approach agree with dogma, or is the hypothesis strong enough to displace dogma?
- What preclinical models exist?
- What competitive approaches exist?
- How would we design a clinical trial?
- What are the PK properties to aim for?
- What experiments need to be done, how much do they cost, how long will they take?
- How will we allocate resources to this?
- Who will be on a scientific advisory board?

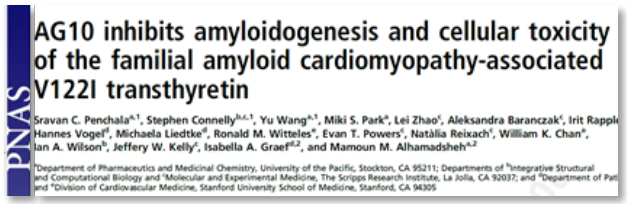
Examples of actions we take

- Site visits
- Survey of the literature, conferences, partnering forums, & grants
- Read literature and become the internal domain experts
- Study epidemiology, genetics, standard of care/ unmet need in the disease space
- Work with the PI on a preclinical plan, and refine the plan with our R&D team
- Conduct interviews with KOLs
- Discuss approach with investment team
- Prepare an investment presentation
- Pitch the opportunity to our board & investment committee

1. Pass	We give detailed feedback and plan to check-in at a later date
2. Hold	We continue conducting diligence to answer outstanding questions
3. Invest	We begin discussing deal terms with institution

Our 2 earliest academic NewCos have seen great success

Academic origins	World-class biotech R&D	Advancement to pivotal trials, value inflection


PNAS

AG10 inhibits amyloidogenesis and cellular toxicity of the familial amyloid cardiomyopathy-associated V122I transthyretin

Sravan C. Penchala[a,1], Stephen Connelly[b,c,1], Yu Wang[a,1], Miki S. Park[a], Lei Zhao[c], Aleksandra Baranczak[c], Irit Rappl
Hannes Vogel[d], Michaela Liedtke[e], Ronald M. Witteles[e], Evan T. Powers[c], Natàlia Reixach[c], William K. Chan[a],
Ian A. Wilson[b], Jeffery W. Kelly[c], Isabella A. Graef[a,2], and Mamoun M. Alhamadsheh[a,2]

[a]Department of Pharmaceutics and Medicinal Chemistry, University of the Pacific, Stockton, CA 95211; Departments of [b]Integrative Structural and Computational Biology and [c]Molecular and Experimental Medicine, The Scripps Research Institute, La Jolla, CA 92037; and [d]Department of Pat and [e]Division of Cardiovascular Medicine, Stanford University School of Medicine, Stanford, CA 94305

Stanford University

Transthyretin stabilizer for familial amyloidosis

Cost: $25M

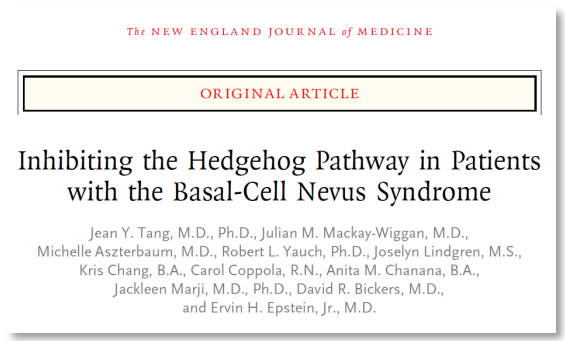
Time: 2 years

MarketWatch

Eidos Therapeutics stock soars in debut

By Emily Bary
Published: June 20, 2018 10:52 a.m. ET

- Now in phase 3


The NEW ENGLAND JOURNAL of MEDICINE

ORIGINAL ARTICLE

Inhibiting the Hedgehog Pathway in Patients with the Basal-Cell Nevus Syndrome

Jean Y. Tang, M.D., Ph.D., Julian M. Mackay-Wiggan, M.D., Michelle Aszterbaum, M.D., Robert L. Yauch, Ph.D., Joselyn Lindgren, M.S., Kris Chang, B.A., Carol Coppola, R.N., Anita M. Chanana, B.A., Jackleen Marji, M.D., Ph.D., David R. Bickers, M.D., and Ervin H. Epstein, Jr., M.D.

UCSF

Topical SMOi for Gorlin Syndrome/ Basal cell carcinoma

Cost: $26M

Time: 3.5 years

FierceBiotech

BIOTECH RESEARCH CRO MEDTECH

Biotech

LEO Pharma inks $760M rare skin disease R&D deal with PellePharm

by Conor Hale | Nov 20, 2018 6:00am

- Now in phase 3 with pharma partner

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Agenda

How we work

How we partner

Portfolio snapshots

AG10 (BBP-265): Potentially most potent transthyretin stabilizer for the treatment of transthyretin amyloidosis

Mechanism of Disease

Native transthyretin circulates in blood as a tetramer

Dissociation into toxic monomers initiates pathogenesis

Monomers aggregate and deposit as amyloid, causing disease
ATTR-CM, ATTR-PN

Impact on patients:

- Progressive, fatal cardiomyopathy (median life expectancy 3-5 yrs) and polyneuropathy
- Both manifestations includes significant disability
- We believe the diagnosed population of ATTR-CM is growing rapidly due to awareness and accurate, non-invasive, diagnostic methods

Mechanism of Drug

AG10 stabilizes TTR tetramer, potentially preventing disease

Dissociation into toxic monomers initiates pathogenesis

Monomers aggregate and deposit as amyloid, causing disease
ATTR-CM, ATTR-PN

AG10 is designed to bind TTR in a way that mimics a naturally-occurring protective mutation

Program Highlights

400k+
ATTR-CM patients worldwide

10k+
ATTR-PN patients worldwide

Clinical status:

Pre-IND	Phase 1	Phase 2	Phase 3

ATTR-CM Ph3 study ongoing (FPI 1Q19)

ATTR-PN Ph3 study expected 2H19

Catalysts:

- Ph2 ATTR-CM OLE data in 2H19
- Ph3 ATTR-CM 12-month data in 2021
- Potential ATTR-CM NDA submission in 2021

Key data:

- Ph2 ATTR-CM data presented in 2H18
  - Normalized serum TTR in all actively treated patients at d28
  - TTR stabilization of ≥90% in all actively treated pts at d28
- Ph3 ATTRibute study initiated in 1Q19
  - Potential registration on 12m 6MWD endpoint, followed by 30m CV outcome/hospitalization endpoint

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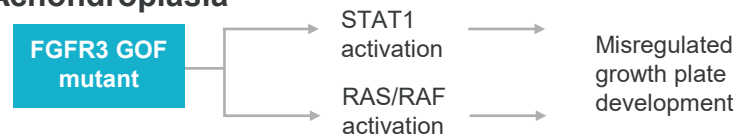
Infigratinib (BBP-831): Only known oral FGFR1-3 inhibitor to treat achondroplasia and FGFR-driven cancers*

Mechanism of Disease

Achondroplasia

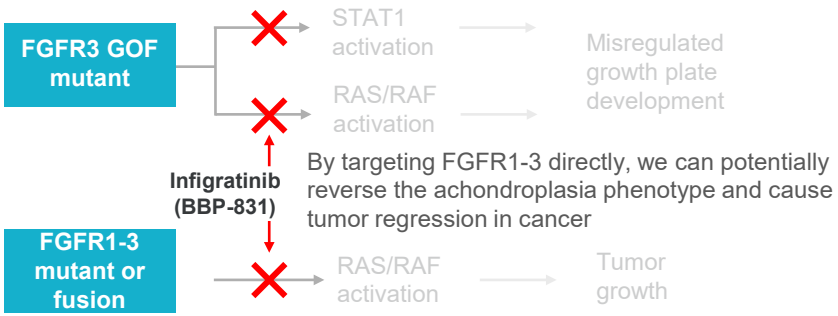


Impact on patients:
- Short stature, spinal compression, narrow foramen magnum
- Increased risk of infant death, sleep apnea, infections
- No currently approved therapies in the US or EU

Oncology

Impact on patients:
- Poor survival, multiple comorbidities that impact QoL

Mechanism of Drug



Program Highlights

55k
Achondroplasia pts in US+EU

37k
Annual new FGFR1-3+ oncology diagnoses in US+EU

Clinical status:

Pre-IND | Phase 1 | Phase 2 | Phase 3

ACH Ph1/2 planned for 2020

Oncology Ph3 enrolling (1L CCA)

Catalysts:

- Achondroplasia IND acceptance and FPI 2020 (expected)
- Achondroplasia clinical data 2021
- Potential oncology NDA in 2020 (2nd line cholangiocarcinoma, CCA)

Key data:

- Achondroplasia:
  - Strong preclinical data with effects on stature, foramen magnum and lumbar disc width in mouse model
  - Only known oral therapy in development
  - Anticipate active dose significantly lower than oncology
- Meaningful clinically data in oncology indications CCA and UC (26.9% and 25.4% ORR respectively)

* Based on clinicaltrials.gov search

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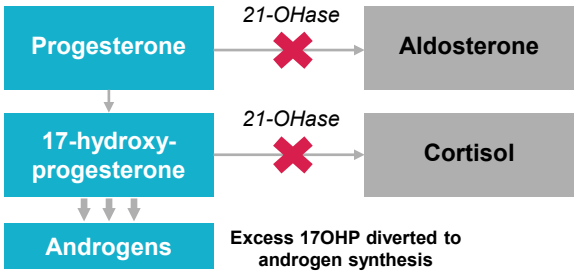
BBP-631: Gene therapy for CAH caused by 21OH Deficiency

Mechanism of Disease

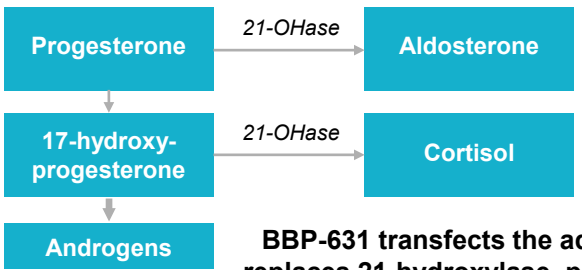
Steroid synthesis pathway



Impact on patients:

- Adrenal crises (can be fatal)
- Lifelong treatment with supraphysiologic steroid, which can cause significant morbidity (CV disease, obesity, bone disease)
- Abnormal sexual development, infertility

Mechanism of Drug



BBP-631 transfects the adrenal glands and replaces 21-hydroxylase, potentially restoring production of aldosterone and cortisol and reducing excess synthesis of androgens

Program Highlights

75K+

Patients with CAH in US+EU

1 / 11,000

Newborns born with CAH

Development status:

Pre-IND	Phase 1	Phase 2	Phase 3

BBP-631

Catalysts:

- IND filing in 2020
- Anticipated clinical proof-of-concept data in 2021

Key info:

- Durability of expression shown in NHP studies; sustained vector copy number and RNA expression out to at least 6 months
- Clinical GMP manufacturing underway at Paragon; in-house process development and analytical capabilities being developed
- Vector construct designed by Dr. Guangping Gao, a world leader in AAV design
- Genotype-phenotype studies show that 5-10% of enzyme activity may be sufficient for clinical impact

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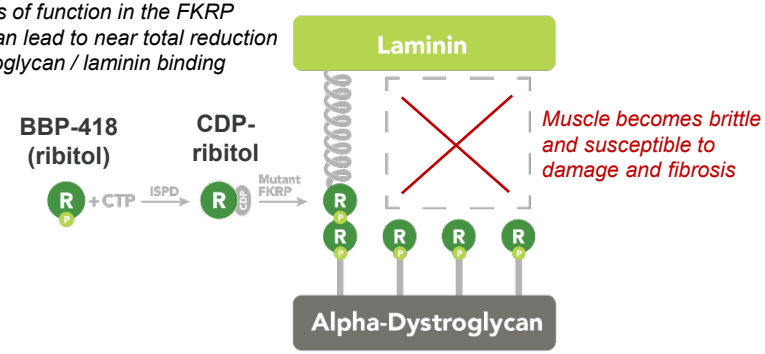
BBP-418: substrate replacement therapy for LGMD2i

Mechanism of Disease

Partial loss of function in the FKRP enzyme can lead to near total reduction in α-dystroglycan / laminin binding

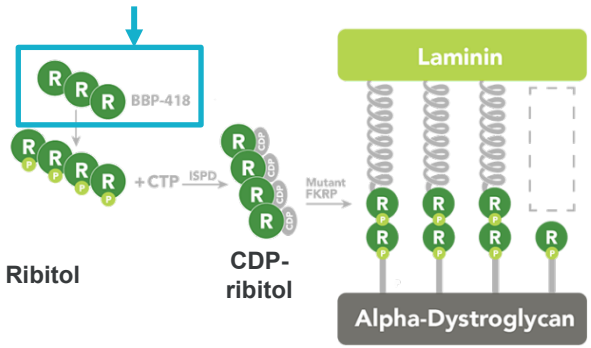


Impact on patients:

- Progressive muscle weakness, leading to loss of ambulation, respiratory function, and cardiac function
- Increased mortality in even the mildest forms of the disease
- No currently approved therapies

Mechanism of Drug

Exogenous BBP-418 (ribitol) partially restores glycosylation



Program Highlights

7000+

LGMD2i pts in US+EU

Development status:

Pre-IND	Phase 1	Phase 2	Phase 3

BBP-418

Catalysts:

- Natural history study start 2H19
- IND filing in 2020

Key info:

- Preclinical tolerability studies of BBP-418 in animals indicates a clean safety profile
- Preclinical studies of BBP-418 in the mouse model of severe LGMD2i (P448L) showed:
  - **Clear BBP-418 uptake in target tissues and efficient conversion into FKRP substrate:** 4x increases in '418 levels in heart and in leg tissue with similar increases in ribitol-5P and CDP-ribitol
  - **Restored *α-dystroglycan* glycosylation in skeletal, cardiac, and diaphragm muscle**
  - **Improved disease pathology and function:** Increase in running time and distance, increase in muscle, decrease in fibrosis, and increase in respiratory function
- **FDA Orphan Drug Designation** for the treatment of LGMD2i

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