

Biopharma Intelligence from Partnering Conferences:

A new source of BD, CI and R&D Strategy Information

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Biotechnology Innovation Organization

PART I

BIO One-on-One Partnering Background & Basics

What is a partnering conference?

- Biotechs* are looking for investors and inlicensors
- Inlicensors and investors are looking for biotechs
- Inlicensors and investors want to meet each other
- Biotechs want to meet with each other
- Academics, Research Institutes, Patient Groups, Vendors, etc. want to meet all the above
- Option A: fly all around the world meeting each other
- Option B: gather at a conference and meet each other

^{*}Biotechs = molecules, devices, diagnostics, platforms, vaccines, digital health, bioinformatics, etc... therapeutic, industrial and agricultural R&D



Where are biopharma deals **BORM?**

"Over 35% of all deals come out of partnering events"



Campbell Alliance's 2014 Dealmaker's Intentions report



"Over 50% of all successful deals come out of partnering events"

Acute need to partner with each other

CMOs, CROs

IT, information

Finance, legal, consulting

Economic development

Biotechs/biopharmas
Larger pharmas
Diagnostics & delivery
Digital Health
Investors

Academia/Tech

Transfer
Hospitals/Institutions

Patient Groups

Suppliers, engineering

Investment banks

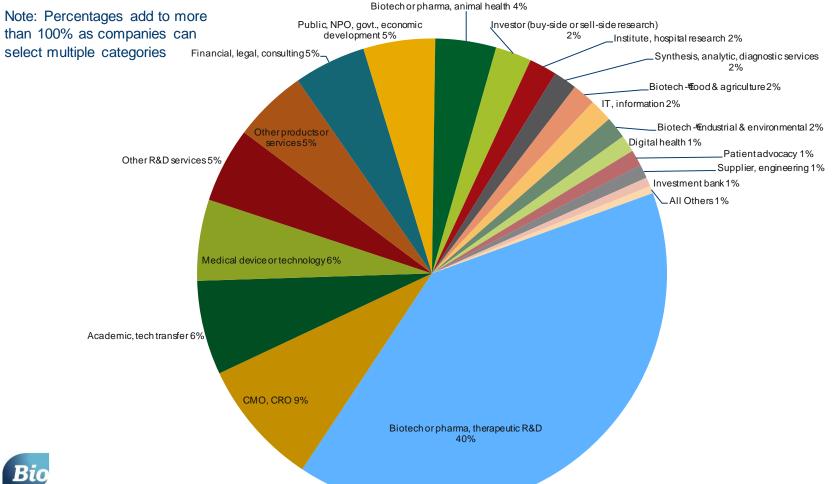
Trade groups

Meet with 'vendors' during free time

Bio

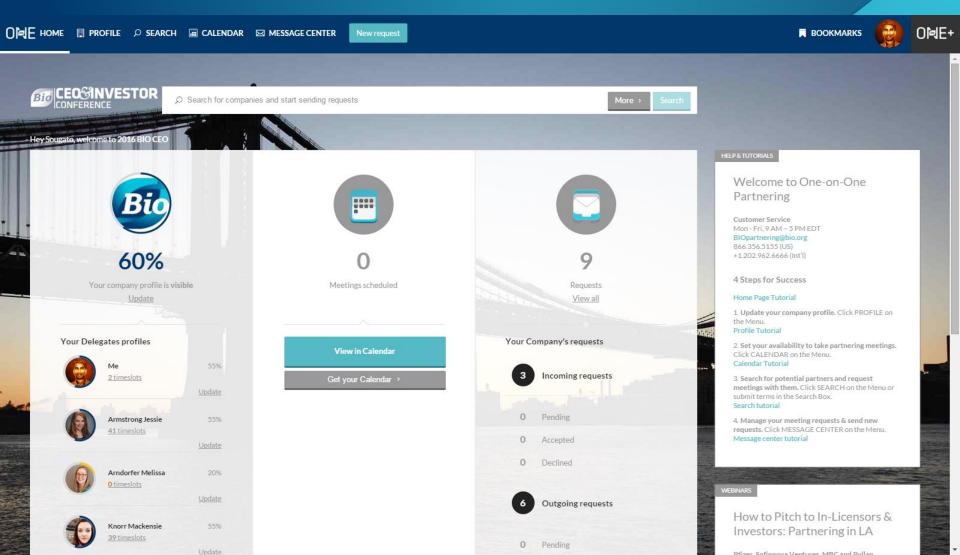
R&D service, other services

2016 Company types overview

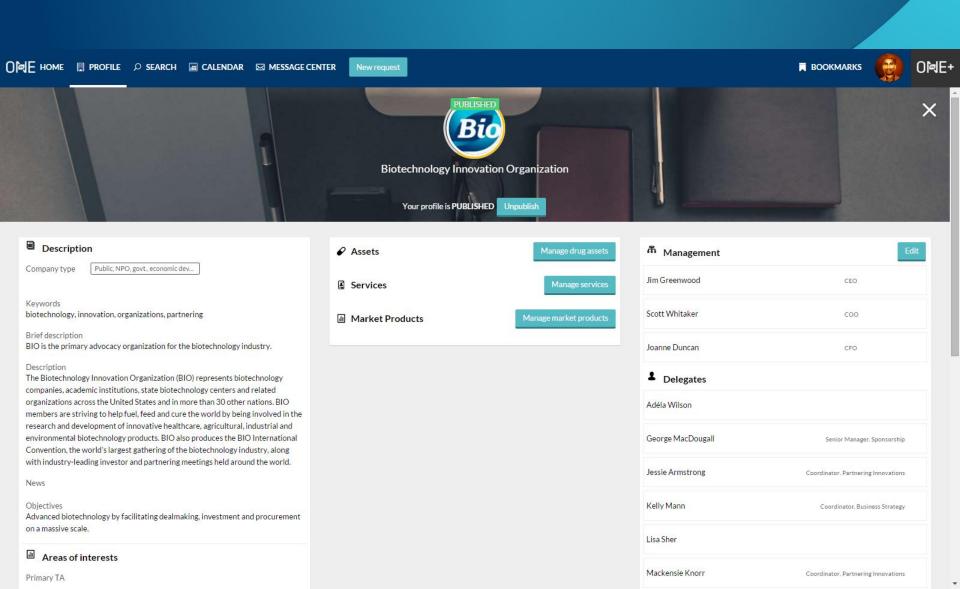




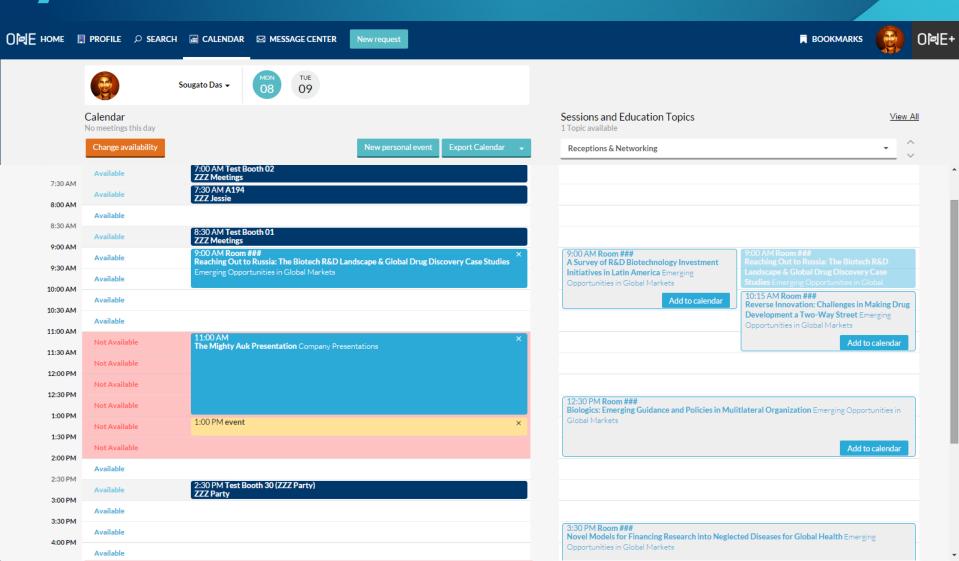
Homepage



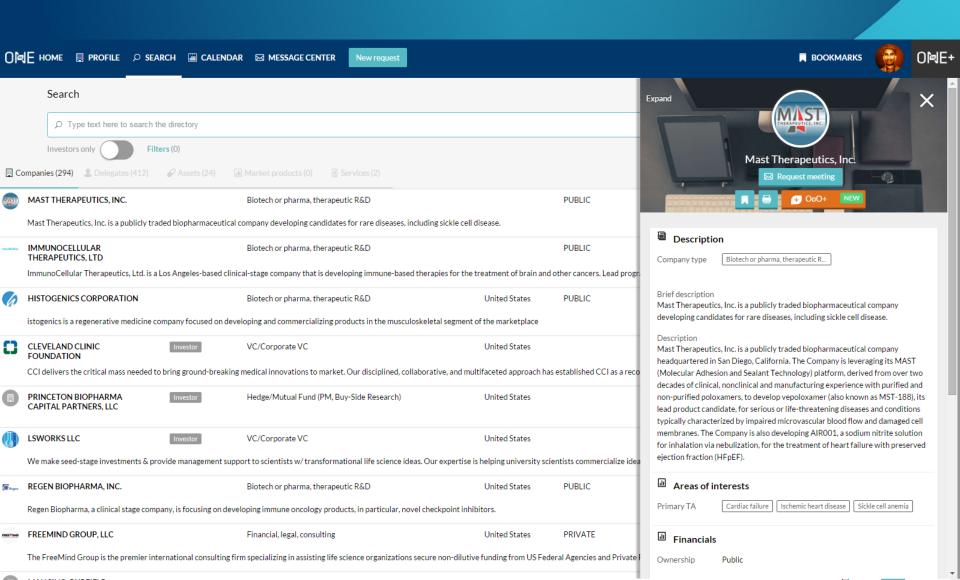
Fill out your company profile



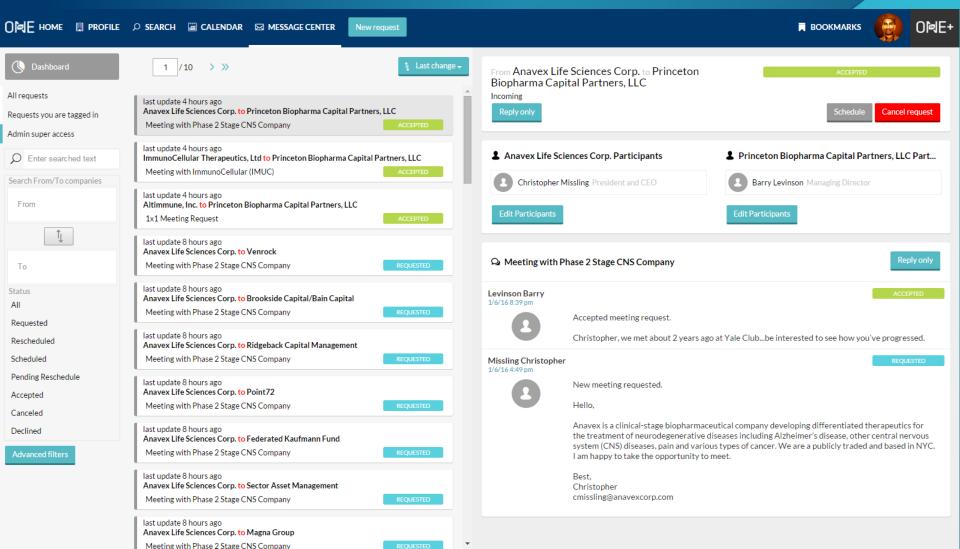
Set your availability and view your schedule



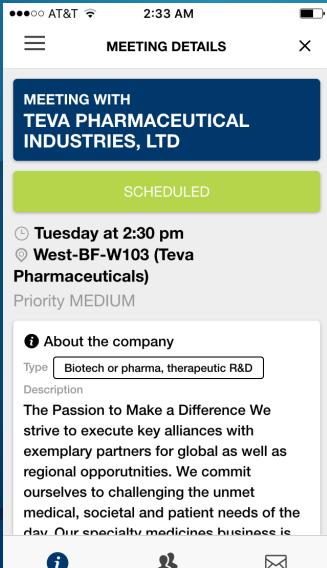
Search for partners



Request, accept and manage meetings



"There's an app for that"











What is BIO

- The Biotechnology Innovation Organization (BIO) is one of the handful of large biopharma advocacy/lobbying organizations. Others include PhRMA, EphRMA and JPMA.
- BIO also covers industrial and agricultural biotechnology.
- BIO finances advocacy/lobbying through membership, partnering conferences and group purchasing programs, with an operating budget of \$75M annually.
- BIO provides the largest partnering conferences in the world, including the BIO International Convention, which hosts nearly 40,000 partnering meetings.
- BIO is expanding partnering services into JP Morgan, Sofinnova, Bio New Jersey and more.



How does conference "partnering" work?

- Company to Company system, not individual to individual
- Populate your company profile
 - Company type(s), description, news, objectives, TAs, HDS
 - Finances
 - Contact info
 - Management and conference delegates
 - Slide decks, 1-pager, publications, videos, data, CDA
- Populate your company's assets
 - Type (antibody/protein/peptide, RNA, cell therapy, etc.)
 - Description, URL, objectives, partners, trials, patents, etc.
 - TAs, phase, MoA, technology (i.e. detailed type)



How does conference "partnering" work? (cont'd)

- Indicate when you are available to meet
- Search for companies and/or assets that interest you
- Send/accept/decline/cancel meeting requests
- BIO schedules meetings (special algorithms maximize number of meetings and minimize distance btw meetings)
- Reschedule or cancel meetings as necessary
- Show up for your meeting. Follow up as necessary.

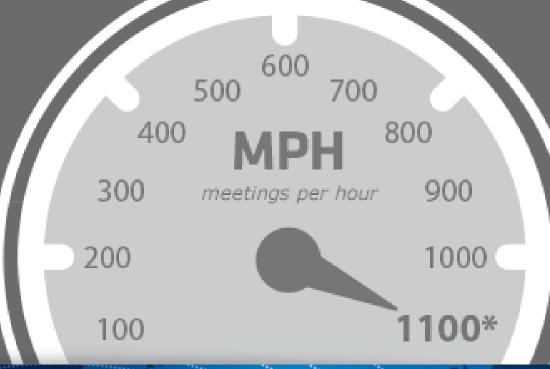
2016 BIO International Convention averaged 1,020 MPH*

*meetings per hour = very clever



2017 BIO Int'l Convention

*Anticipated 1,100 Meetings Per Hour



PARTNERING WEBINAR Wed. April 5, 2pm

Learn what BIO One-on-One Partnering can do for your busines and how to be successful at BIO 2017 in San Diego!





June 19-22, 2017 San Diego Convention Center San Diego, CA



System stats, per day

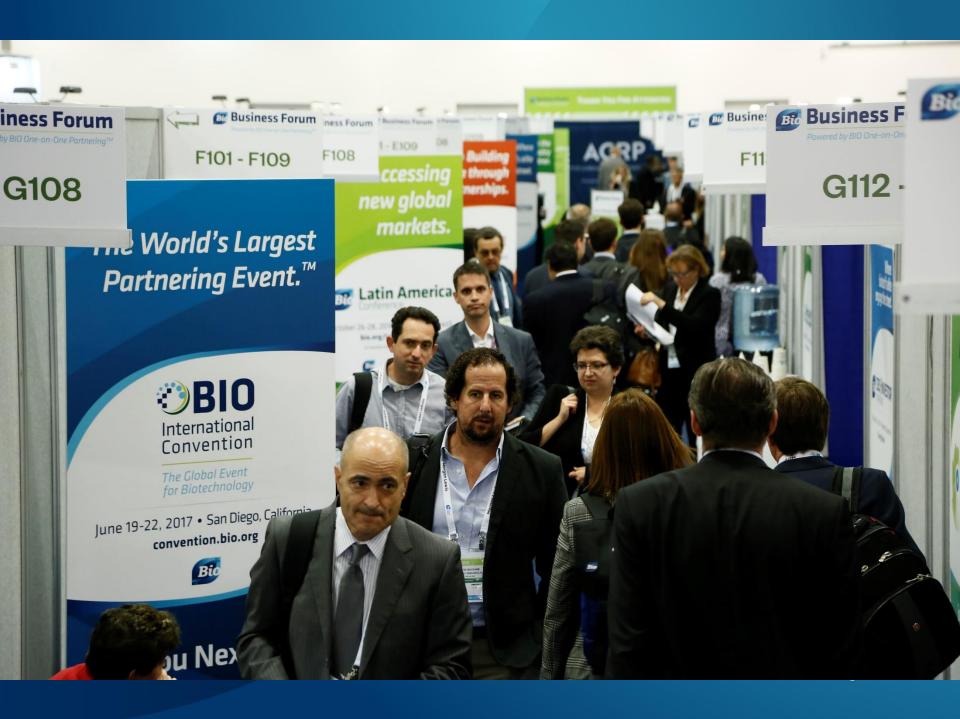
Daily usage:

- 14,000+ visits
- 5,600 unique visitors
- 20 minute average visit duration
- 162,600 page views

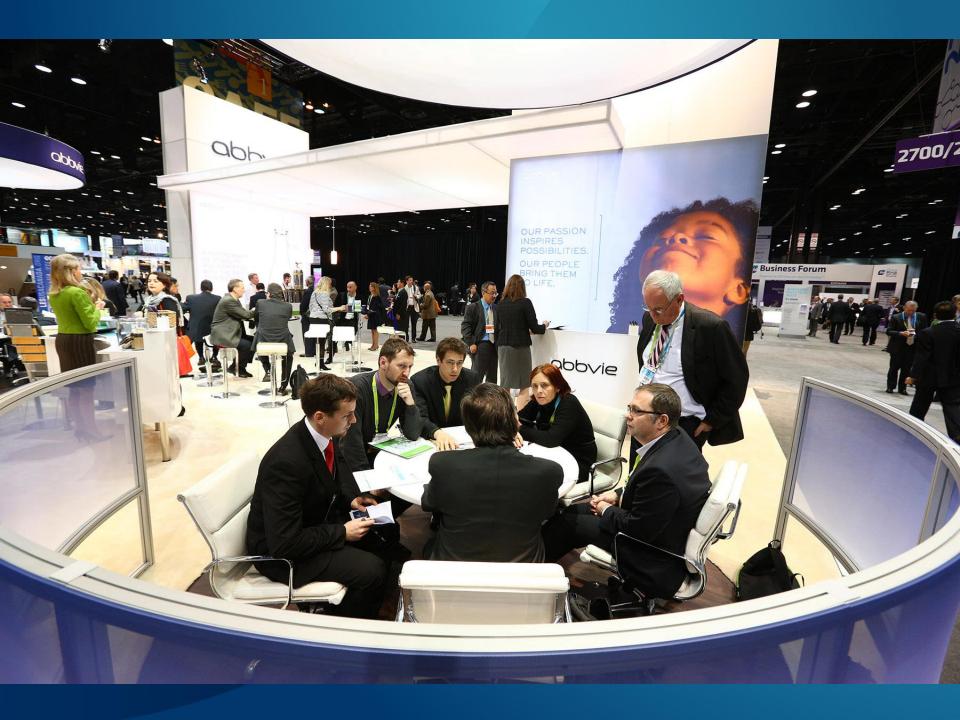












Monday (half-day of partnering)



Tuesday

7:30 AM	Available	7:00 AM South-BF-N101 Landsteiner Scientific S.A. de C.V.
8:00 AM	Available	7:30 AM South-BF-N101 HERON THERAPEUTICS, INC.
8:30 AM	Available	8:00 AM South-BF-N105 XEDITON PHARMACEUTICALS INC.
	Available	8:30 AM West-BF-T116 BioSyent
9:00 AM	Available	9:00 AM South-BF-P118 Eurofarma Laboratórios SA
9:30 AM	Available	9:30 AM South-BF-P118 Medicure, Inc
10:00 AM	Available	10:00 AM West-BF-YY121 Valeo Pharma Inc
10:30 AM	Available	10:30 AM West-BF-YY121 Sandoz Biopharmaceuticals
11:00 AM	Available	11:00 AM West-BF-YY121 Kowa Company, Ltd.
11:30 AM	Available	11:30 AM West-BF-YY117 Knight Therapeutics Inc.
12:00 PM	Available	12:00 PM South-BF-P118 DKSH
12:30 PM	Available	12:30 PM West-BF-YY121 Teijin Pharma Limited
1:00 PM	Available	1:00 PM West-BF-YY121 Cipla Ltd.
1:30 PM	Available	1:30 PM West-BF-YY121 Novartis Pharmaceuticals Corporation
2:00 PM	Available	2:00 PM West-BF-YY121 Camurus AB
2:30 PM	Available	2:30 PM South-BF-O115 Paladin Labs, Inc
3:00 PM	Available	3:00 PM South-BF-P118 Exagen Diagnostics. Inc

3:30 PM 4:00 PM	Available	3:30 PM West-BF-ZZ120 Covance, Inc
4:00 PM	Available	4:00 PM West-BF-Y130 CSL Behring
5:00 PM	Available	4:30 PM South-BF-D101 Biodelivery Sciences International, Inc.
5:30 PM	Available	5:00 PM South-BF-N103 Jerusalem Pharmaceuticals
6:00 PM		5:30 PM meet TELESTA THERA AT MARRIOTT MA
6:30 PM		6:00 PM MEET PHOSPHAGENICS AT MARRIOT M
7:00 PM		6:30 PM meet dave penake from Saol thera at man
7:30 PM		Non partnering hours
8:00 PM		Non partnering hours
8:30 PM		Non partnering hours
1/2		



LIBBS Farmaceutica Ltda

3:00 PM South-BF-P102

Lacer, SA

Lee's Pharmaceutical Holdings Ltd.

2:30 PM North-EX-5202h (Hong Kong Pavilion)

Available

Available

Available

2:30 PM

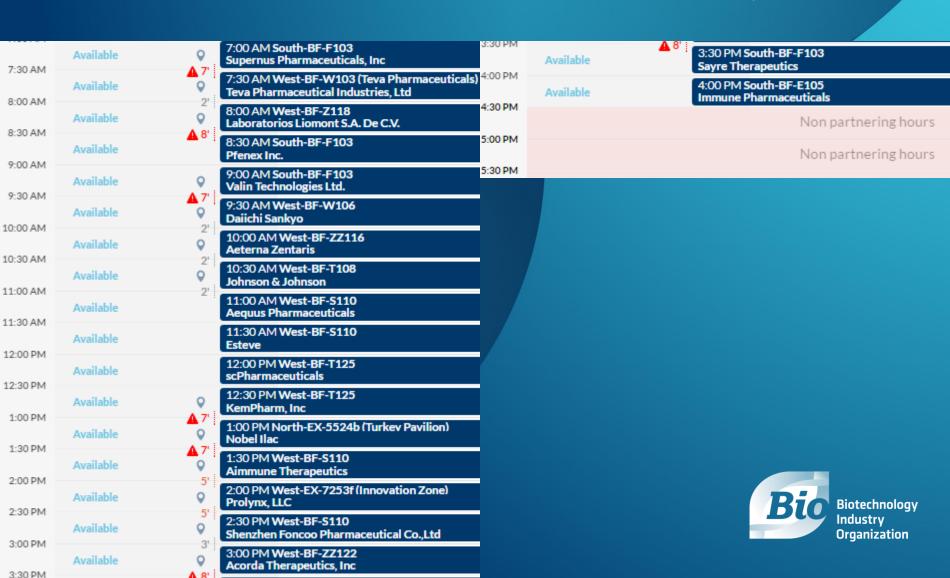
3:00 PM

Wednesday

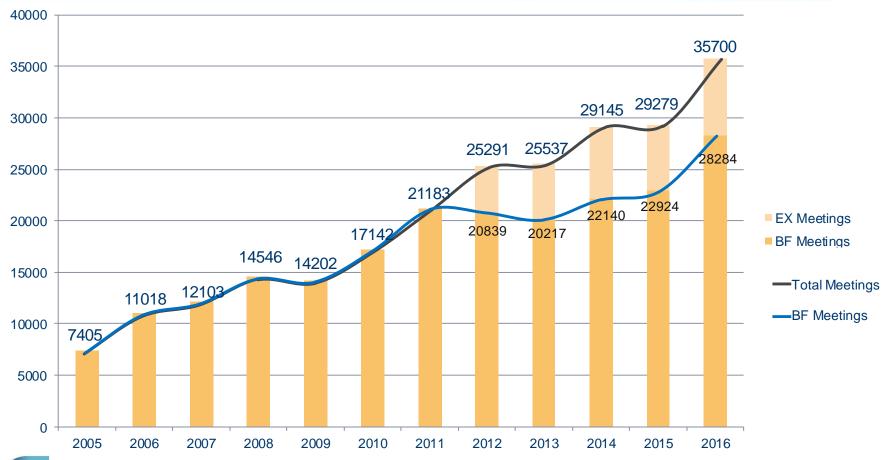
7:30 AM	Available		7:00 AM South-BF-D101 Novum Pharma	3:30 PM	Available	6'	3:30 PM North-EX-6452b (Argentina Pavilion) BIOPROFARMA
8:00 AM	Available		7:30 AM South-BF-D101 Marathon Pharmaceuticals	4:00 PM	Available	5'	4:00 PM South-EX-905d (German Pavilion) IIS Innovative Injektions-Systeme GmbH & Co. KG
8:30 AM	Available		8:00 AM South-BF-D101 Grupo Biotoscana	4:30 PM	Available		4:30 PM South-BF-Q101 Clinigen Group - Business Forum
9:00 AM	Available	Q ∆ 8'	8:30 AM South-BF-D101 Phebra Pharma	5:00 PM	Available		5:00 PM South-BF-O101 IMPAX Laboratories, Inc
9:30 AM	Available	Q 2'	9:00 AM West-BF-Y114 Verastem	5:30 PM			5:30 PM MEDIVIR AT MARRIOT MARQUIS HOTE
10:00 AM	Available	2 ;	9:30 AM West-BF-U106 SANOFI	6:00 PM			6:00 PM meet baver at marriott marguis Mr Huan
10:30 AM	Available	Q 8'	10:00 AM West-BF-U107 Biomm	6:30 PM			Non partnering hours
11:00 AM	Available	A • ;	10:30 AM South-BF-Q101 Amneal Pharmaceuticals, LLC	7:00 PM			Non partnering hours
11:30 AM	Available		11:00 AM South-BF-O101 Grupo AVE	7:30 PM			Non partnering hours
12:00 PM	Available		11:30 AM South-BF-Q108 NuvOx Pharma, LLC	8:00 PM 8:30 PM			Non partnering hours
12:30 PM	Available		12:00 PM South-BF-O101 JSC Pharmasyntez	8.30 PM			
1:00 PM	Available		12:30 PM South-BF-Q101 Deva Holding				
1:30 PM	Available	Q 2'	1:00 PM South-BF-O101 ALVOGEN				
2:00 PM	Available	2	1:30 PM South-BF-B109 Kineta, Inc				
2.00 F14		_	2:00 PM South-BF-Meeting Point SS				Piotochnology



Thursday

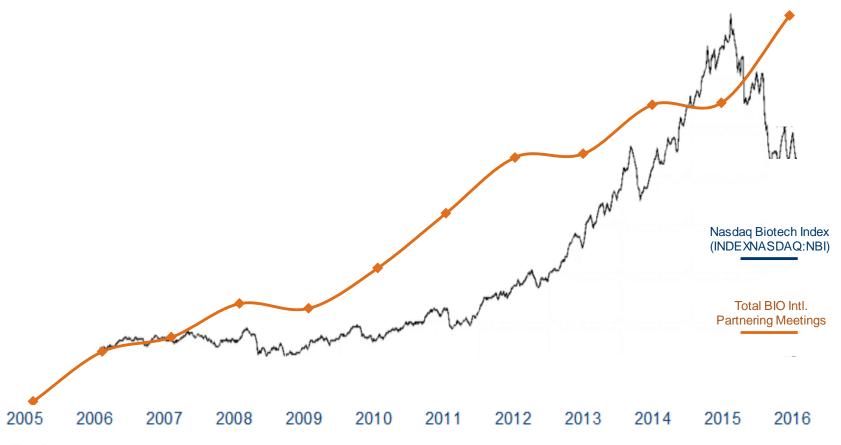


One-on-One Partnering *Growth*





One-on-One Partnering vs. NBI





One-on-One 3-yr overall trend

Event Totals	2014 Partnering	2015 Partnering	2016 Partnering
Total requests	153,093	156,095	190,520
Scheduled meetings	29,145	29,279	35,700
Companies	3,160	3,144	3,517
Countries	55	52	54
Meetings per company	18.4	18.6	20.4



BIO 2016 Top 10 Cos. (in alpha order)

- Abbvie
- Bayer
- Daiichi Sankyo
- **J&J**
- JT Pharma
- Merck
- Novartis
- Pfizer
- Roche
- Sanofi



BIO partnering expanding...

BIO One-on-One Partnering powers...

- BIO International Convention: 3500+ companies
- BIO Partnering @ JPM: 2000+ companies and growing fast
- BIO CEO & Investor Conference: 800+ companies
- BIO Investor Forum: 500+ companies
- BIO Asia: 200+ companies
- BIO Latin America: 300+ companies
- Sofinnova conferences: 100+ companies
- French partnering events including Lyon Biopole, MedStartUp, Biovision: 500+ companies
- BIO state affiliate conferences with BioNJ and others in pipeline...



Biotechnology Innovation Organization

PART II

Information Assets in BIO One-on-One Partnering vs.

Top Pipeline DBs

Top-level summary of assets

Longitudinal data on:

- Name, titles, contacts of relevant BD personnel
- Company* profiles
- Asset profiles
- Meeting trends
- Meeting request templates
- Meeting success ratios
- Interests (programming, presentations, survey)

*"Company" used generically to mean company, university, foundation, organization, association, etc.



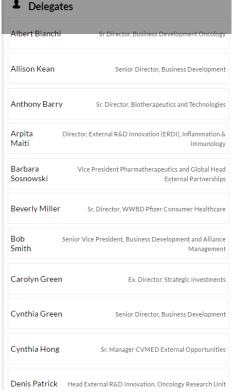
Name, titles, contacts of relevant BD personnel

- History of job changes
- Role relative to organization (most pipeline DBs list a single contact for everything; countless BD people have told me that comms going to their generic BD contact go nowhere)
- Ability to contact through BIO One-on-One Partnering API (similar to linkedin – send "inmail" without revealing email)
- Geography



Name, titles, contacts of relevant BD personnel (example)

Pfizer (BIO, with messaging API)



40+ BD contacts



Pfizer (pipeline DB)
Ms Polly Murphy
R&D Business Dev.
pollymurphy@pfizer.com



Name, titles, contacts of relevant BD personnel (example)

- Glycomimetics (BIO, with messaging API)
 - Armand Girard, Vice President, Corporate Development
 - David Entin, Director, Corporate Contracts, Licensing & BD
 - Rachel King, CEO
- Glycomimetics (pipeline DB)
 - webmaster@glycomimetics.com



Company* profiles

- New companies and updates to existing companies
- What is the company trying to do?
- TAs, from the internal perspective
- Finances and size by # of employees
- Do affiliates/subsidiaries act independently when partnering?
- How does a company actually segment its businesses?
- Slide decks, 1-pager, publications, videos, data, CDA
- 8% 10% exclusivity to BIO DB (for full phase, US conf)
- Other types "out of remit" therapeutic R&D companies
- You need R&D services and tech too, BIO has those cos.
- *"Company" used generically to mean company, university, foundation, organization, association, etc.



2017 JPM Company Profile - Exported on March 30 2013





1ST BIO

South Korea Biotech or pharma, therapeutic R&D

Alzheimers disease, Cancer

FINANCIALS

Employees Year Founded Ownership Last Funding Total Funding 10 to 50 2016 PRIVATE Seed Round,A Round

CONTACT

(031) 8023-5332 http://www.1stbio.com/

Page 1 of 2

Brief description

IST BIO is a preclinical-stage biotech company based in Korea and San Francisco, focused on developing breakthrough therapies in neurodegenerative diseases, immuno-oncology and rare diseases.

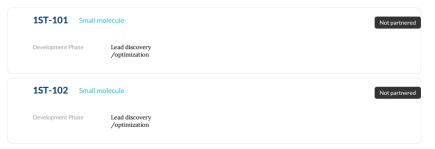
Description

IST BIO is dedicated to developing novel therapies with a key value to provide new treatment options to patients, by working through a new way of open innovation and by employing extensive risk mitigation strategies even in early phase drug discovery. Our main focus is currently NCE development in neurodegenerative diseases, immuno-oncology and rare diseases.

Delegates

Jamie Jae Eun Kim

Assets





2017 JPM Company Profile - Exported on March 30 2017





CENSA PHARMACEUTICALS INC

United States Biotech or pharma, therapeutic R&D

Amino acid and protein metabolism disorder, Phenylketonuria, Cofactor deficiency

FINANCIALS

Year Founded Ownership 2015 PRIVATE

CONTACT

☑info@censapharma.com €617-225-7700

△ http://www.censapharma.com/

Brief description

Censa Pharmaceuticals is a venture backed company developing novel therapies for orphan disorders.

Description

Censa Pharmaceuticals Inc. is a venture backed company developing novel therapies for people living with Phenylketonuria and Primary BH4 deficiency. Earlier stage programs target neuropsychiatric conditions. Censa's lead product is an orally available, small molecule, naturally occurring NCE pro-drug of an approved therapy which is regulated in the US under the 505b2 route. The company will file an IND by mid 2017. FDA approval for its first clinical indication is expected in early 2019.

Delegates

Jonathan Reis

Page 1 of 1



2017 JPM Company Profile - Exported on March 30 2017





HEPATX CORPORATION

United States Biotech or pharma, therapeutic R&D

Liver disease

FINANCIALS

Employees Year Founded Ownership Less than 10 2015 PRIVATE

CONTACT

Page 1 of 1

Brief description

Hepatx is an early stage (preclinical) company out of Stanford that is developing cell therapies for liver disease, a highly underserved and cost intensive market.

Description

Hepatx is developing hepatocyte cell therapies to support liver function as an alternative to costly and often unavailable transplants. Our proprietary process manufactures liver cells from discarded lipoaspirate. One standard liposuction can produce enough hepatocytes to replace an entire human liver, allowing us to meet the huge demand. Our technology provides an off-the-shelf therapy by banking manufactured hepatocytes for administration on-demand for the thousands of liver failure patients in need. We are initiating manufacturing development and plan to start clinical studies in 2 years. Our core IP is licensed from Stanford and we are developing a comprehensive IP portfolio. Our team has extensive experience from Stanford University and biotech/pharma in pre-clinical, clinical, and manufacturing development and is well positioned to execute this program.

Objectives

Seeking value-add investors and advisors

Delegates

Eric Schuur President/CEO



2017 JPM Company Profile - Exported on March 30 2017





TRPHARM

Turkey Biotech or pharma, therapeutic R&D

Genetic disorder,Immune disorder,Infectious disease, Inflammatory disease,Neoplasm,Enzyme metabolism disorder,Metastasis,Anesthesia

FINANCIALS

Year Founded Ownership 10 to 50 2013 PRIVATE

CONTACT

△ http://www.trpharm.com/?lang=en

Brief description

TRPharm is a pharma company focused on innoviation with investments into R&D and a cutting edge biotechnological manufacturing capability with the aim of becoming a multinational player in the MENA region based out of Turkey.

Description

Our business is focused on 3 pillars: 1) Conducting pre-clinical and Phase I, Phase II and Phase III clinical research activities for our propriatory products and co-development partnerships, 2) Commercial value generation in our partnerships with compliance to highest ethical standarts and continued contribution to science and education, 3) Carrying out biotechnological manufacturing at our own facility in high class standards that hosts top level technologies and to to fulfill domestic / foreign demands.

News

http://www.trpharm.com/?lang=en http://www.nasdaq.com/article/dr-reddys-trpharm-collaborate-for-manufacture-of-biosimilar-drugs-in-turkey-20160311-00036

Objectives

TR-Pharm is actively seeking licensing and co-development partnerships in late stage products that will complement its existing propriatory and inlicensed portfolio. Our portfolio is led by oncology, infectous diseases, inflammatory, hospital products and rare diseases.

Delegates



Page 1 of 1



Cell Care Therapeutics

- BIO: "Cell Care Therapeutics is a preclinical stage company developing first-in-class mesenchymal stem cell derived biopharmaceuticals to treat inflammatory and degenerative ophthalmic diseases."
- Pipeline DB: "Cell Care Therapeutics is a privately-held company focus on the development of stem cell-based therapies for the treatment of degenerative and inflammatory diseases of the eye"
- BIO: Phase = Target id/valid | Lead disc/opt | Preclin | P1 | P2 | P3 | Pre-Reg | Reg | Market
- Pipeline DB: Disc | P1 | P2 | P3 | Pre-Reg | Reg | Launched



Cell Care Therapeutics (cont'd)

- BIO: TA = Degeneration, Inflammation, Ocular disease
- Pipeline DB: TA = Blindness, Cancer, Ocular disease, Ocular inflammation
- BIO: Employees = <10; Founded = 2014; Seed round funding of \$1.8M
- Pipeline DB: 6 patents, 1 drug, 1 deal
- BIO: Veronique Jotterand, M.D., CMO; Rajashekhar Gangaraju, Ph.D., CSO; David McQuillan, Ph.D, R&D Strategist; Nicolas Sohl: nsohl@cell-care.com



Pipeline DB: info@cell-care.com



Progenra

- Primary indications in BIO are various cancers and others are secondary. In pipeline DB (PDB) most TAs mgmt. said were secondary are primary. No cardio, lipid metab, obesity listed in BIO. Missing in PDB are arthritis and granularity on which cancers.
- Similar to last example, BIO has better contact/management information, including detailed background on CEO (with pic), and PDB has more details on all drugs, patents and deals.
- Progenra has 20+ drugs in PDB. It has 2 in BIO. The 2 in BIO are the partnering leads.



Progenra (cont'd)

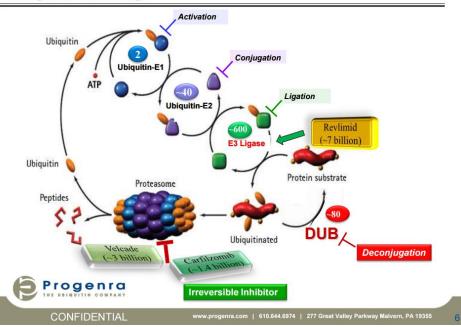
- Progenra description in PDB = 50+ words summarizing tech, TAs, that co seeks outlicensing, and 1 deal.
- Progenra description in BIO = 680+ words detailing company, news and recent conferences where presented, and specific partnering objectives.
- BIO: PDF of company presentation deck
- BIO: One-Pager ppt
- BIO: Hi-Res logo
- BIO: Publication PDF (Elsevier BV)
- BIO: Publication PDF (nature medicine)



Progenra (cont'd)

BIO: PDF of company presentation deck

Ubiquitin Pathway





Progenra (cont'd)





Seeking Funding for Clinic

Novel Immune oncology

Company Description

Progenra's mission is discovering and developing first in class novel medicines based on the ubiquitin-proteasome pathway (UPS) and addressing major unmet medical needs.

Therapeutic Focus:

- Pathway and platform rather than single target. Enabling the study of multiple diseases that represent major markets:
- Cancer/Immune oncology, Cystic Fibrosis, Inflammatory diseases
 Company Background
- Integrated R&D organization, spanning target validation, assay development, high throughput screening, medicinal chemistry and to pre-clinical PoC.
- Efficient Drug Discovery Platform, Medicinal Chemistry, lead optimization to the clinic
- USP7 drug in late preclinical development targeting immune oncology

Stage of Immune Oncology Drug Development

Preclinical: Ready to file

ND

Ready for Clinic

- Hits identified from our small molecule library have been optimized. Highly potent and selective USP7 inhibitors block tumor growth in animal models.
- USP7 compound synergies with check point biologicals
- DMPK and formal toxicology studies in progress

http://www.progenra.com/ Malvern, PA

Butt@progenra.com

Lead Indication / Market Size

- Immunoevasion is a hallmark of cancer and plays an important role in tumor growth by impairing the immune system.
- IO antibodies blocking immunoevasion, launched as Yervoy, Opdivo and Keytruda, target melanoma, NSCLC; additional indications underway
- T-cell therapy market projected at USD 30 billion by 2030, expanding at annualized growth rate of >100% (BioMarket)
- It is recognized that small molecule combinations with IO agents will
- provide increased efficacy and cost effectiveness
- Numerous combination trials of IO antibodies + small molecules are ongoing for a variety of tumor types (solid and hematological)

Value Proposition / Differentiation/ Regulatory Path

- Progenra's therapy and technology is a novel approach to several disease implications
- USP7 inhibitor drug has value over biological single agents for its low cost of goods, reduction of effective biological agent concentration (potentially increasing safety), novel dual mechanism of action employing IO and, unlike other small molecule combination candidates, direct tumor eradication

Management Team

- · Progenra Team: Seasoned team with big pharma experience
- Tauseef R. Butt, PhD- President & CEO (NIH, SmithKline/GSK)
- Michael Mattern, PhD- VP (Corporate Affairs SmithKline/GSK)
- · Joseph Weinstock, PhD- VP (Medicinal Chem- SmithKline/GSK)
- David Newman Ph.D- VP (Natural Product Med Chem, SKF/NIH)



Progenra (cont'd)

BIO: Publications



HAUSP deubiquitinates and stabilizes N-Myc in neuroblastoma

Omid Tavana^{1,2}, Dawei Li^{1,2}, Chao Dai^{1,2}, Gonzalo Lopez¹⁻⁴, Debarshi Banerjee^{2,5}, Ning Kon^{1,2}, Chao Chen^{6,7}, Andrea Califano^{1-4,8}, Darrell J Yamashiro^{2,5}, Hongbin Sun^{6,7} & Wei Gu^{1,2,9}

The MYCN proto-oncogene is amplified in a number of advanced-stage human tumors, such as neuroblastomas. Similar to other members of the MYC family of oncoproteins, MYCN (also known as N-Myc) is a transcription factor, and its stability and activity are tightly controlled by ubiquitination-dependent proteasome degradation^{1–4}. Although numerous studies have demonstrated that N-Myc is a driver of neuroblastoma tumorigenesis, therapies that directly suppress N-Myc activity in human tumors are limited. Here we have

of several cellular factors^{8–17}. Nevertheless, it remains unclear which cellular factor(s) might contribute to the neonatal lethality of Nes-Cre;Usp7^{GUII} (hereafter referred to as Nes-Cre;Hauspf^{IUII}) conditional-knockout mice¹⁸, in which Cre recombinase, expressed under the control of the nestin gene (Nes) promoter, mediates deletion of loxP-flanked (floxed) Hausp exon 6, resulting in production of a truncated catalytically inactive protein. Here we sought to identify novel substrates of HAUSP that are outside of the p53 network and that specifically control embryonic cell growth and development of



Progenra (cont'd)

BIO: Publications



Contents lists available at ScienceDirect

EBioMedicine

journal homepage: www.ebiomedicine.com



Research Paper

Ubiquitin-specific Protease-7 Inhibition Impairs Tip60-dependent Foxp3 + T-regulatory Cell Function and Promotes Antitumor Immunity

Liqing Wang ^a, Suresh Kumar ^b, Satinder Dahiya ^a, Feng Wang ^b, Jian Wu ^b, Kheng Newick ^c, Rongxiang Han ^a, Arabinda Samanta ^a, Ulf H. Beier ^d, Tatiana Akimova ^a, Tricia R. Bhatti ^a, Benjamin Nicholson ^{b,1}, Mathew P. Kodrasov ^b, Saket Agarwal ^b, David E. Sterner ^b, Wei Gu ^e, Joseph Weinstock ^b, Tauseef R. Butt ^b, Steven M. Albelda ^c, Wayne W. Hancock ^{a,*}

- * Division of Transplant Immunology, Department of Pathology and Laboratory Medicine, Children's Hospital of Philadelphia and Perelman School of Medicine, University of Pennsylvania, Philadelphia, PA 19104, USA
- b Progenra, Inc., Malvern, PA 19355, USA
- * Pulmonary, Allergy & Critical Care Division, Perelman School of Medicine, University of Pennsylvania, Philadelphia PA19104, USA



Asset profiles

- New assets and updates to existing assets
- Alternate names
- What is the company trying to do with the asset?
- TAs, MoAs, technologies, from the internal perspective
- Which assets are live, which are dead, reality vs. "NDR"
- Futures e.g. phase planned for 2018
- 5% 6% exclusivity to BIO DB (for late phase, US conf)
- Other types "out of remit" assets (e.g. drug delivery)
- Slide decks, 1-pager, publications, videos, data



Asset missing examples (out of 1st 50 drug asset results)

2017 BIO CEO Company Profile - Exported on March 31 2017





SPARK THERAPEUTICS

United States Biotech or pharma, therapeutic R&D

Huntingtons chorea, Blood clotting factor deficiency, Mitochondrial disease, Neurodegenerative disease, Retinopathy, Retinitis pigmentosa, Choroideremia

FINANCIALS

Employees 50 to 200
Year Founded 2013
Ownership PUBLIC
Ticker ONCE
Market Cap. 1600.0
Last Funding B Round

CONTACT

Brief description

Spark is a gene therapy leader seeking to transform the lives of patients with debilitating genetic diseases through the delivery of life-altering treatments.

Delegates

Katherine High

Co-Founder, President, and Chief Scientific Officer

Clinical Indications

Assets



Biological therapeutic



Huntingtons chorea

Asset missing examples (out of 1st 50 drug asset results) (cont'd)

2017 BIO CEO Company Profile - Exported on March 31 2017



Not partnered

Not partnered



SPARK THERAPEUTICS

United States
Biotech or pharma, therapeutic R&D

Huntingtons chorea, Blood clotting factor deficiency, Mitochondrial disease, Neurodegenerative disease, Retinopathy, Retinitis pigmentosa, Choroideremia

FINANCIALS

Employees 50 to 200
Year Founded 2013
Ownership PUBLIC
Ticker ONCE
Market Cap. 1600.0
Last Funding B Round

CONTACT

Leber Hereditary Optic Neuropathy (LH Gene therapy

Spark Therapeutics is investigating a potential gene therapy for LHON, a mitochondrial disease that can cause the optic nerve to atrophy, leading to sudden, severe loss of central vision. The prevalence of LHON in most populations is not clearly understood. Based on EU prevalence, it is estimated that LHON affects more than 7,000 patients in the U.S. and more than 160,000 globally.

Development Phase
Clinical Indications

Preclinical testing

Mitochondrial disease;

Retinopathy

Mechanism of Action

Gene therapy
Biological therapeutic

modulator

RHO-adRP

Gene therapy

Spark Therapeutics is developing RhoNova, an investigational gene therapy for rhodopsin-linked autosomal dominant retinitis pigmentosa (RHO-adRP), an inherited retinal disease (IRD) that leads to visual impairment and in the most severe cases, to blindness. RhoNova is being developed to both potentially suppress the expression of

a faulty gene and to deliver normal copies of the RHO gene, with the goal of restoring normal expression.

Development Phase Preclinical testing Mechanism of Action Channel rhodopsin gene

Clinical Indications Retinitis pigmentosa Technologies Biological therapeutic

SPK-7001: Choroideremia Gene therapy

Not partnered

Spark Therapeutics is advancing an open-label, dose-escalating Phase 1/2 trial designed to assess the safety and preliminary efficacy of subretinal administration of investigational SPK-7001. Choroideremia (CHM) is an X-linked inherited retinal disease (IRD) that usually manifests in affected males during childhood as night blindness and a reduction of visual field, followed by progressive constriction of visual field, ultimately leading to complete blindness.



Asset (& co) missing examples (out of 1st 50 drug asset results) (cont'd)

2017 BIO CEO Company Profile - Exported on April 01 2017



We are seeking indications of interest to participate in a \$1.5

MM financing to advance our lead candidate through IND studies, with a quick pivot to a \$5-\$7MM financing to fund a



TENSUS THERAPEUTICS

United States Biotech or pharma, therapeutic R&D

Diabetic eye disease, Retinopathy, Diabetic retinopathy, Diabetic macular edema

FINANCIALS

Employees Year Founded Ownership Less than 10 2014 PRIVATE

CONTACT

https://www.tensustherapeutics.com/

Delegates

Christopher Stanley

Co-Founder & Managing Member

Assets

TT-001 Small molecule

Not partnered

The "holy grail" of topical ocular dosing is the ability of a formulation to penetrate the outer layers of the eye and safely provide therapeutic concentrations to target tissue in the retina. We have licensed exclusive rights to a patented, thermodynamically stable microemulsion formulation of fenofibrate. Our rights include all therapeutic indications, via any route of administration, and our lead topical ocular formulation is covered by two issued US patents with the international application entering national phase filings. Our animal data demonstrate that topical dosing of our lead formulation provides a 15X increase in retinal concentrations of fenofibric acid (the active metabolite of fenofibrate) compared to oral dosing; additional nonclinical studies are ongoing. We have also completed a non-GLP 30-day rabbit toxicology study with four-times-a-day dosing with our lead formulation with no toxicity. These observations, coupled with the extensive body of published nonclinical and clinical safety data for fenofibrate, indicate a high probability of technical success. For our development plan and approval pathway, we intend to employ an efficient 505(b)(2) strategy, which will allow us to reference published literature data demonstrating the systemic safety of fenofibrate.

Objectives

Phase II clinical trial.

Development Phase

Preclinical testing

Mechanism of Action Technologies

PPAR modulator

Diabetic eye disease; Retinopathy;Diabetic retinopathy;Diabetic macular edema Small molecule therapeutic



Asset incomplete examples (out of 1st 50 R&D drug results)

4-1BB CTL (company = Eutilex)

Pipeline DB: Indexes like TA, MoA, Phase, etc.

...developing adoptive T cell therapy for cancer; P 1/2a trial; 7/16: IND P 2b; 3/14: S.Korean P 1/2a trial approved; 12/12: S.Korean P 1 trial approved; 4/10: S.Korean trial began; 3/14: trial ended, safe/effective for EBV benign tumor



Asset incomplete examples (out of 1st 50 R&D drug results) (cont'd)

BIO: Indexes like TA, MoA, Phase, etc.

...autologous procedure uses 50ml of patient's blood & takes 2 weeks for prep to re-infuse into the patient. Clinical POC achieved: complete, durable remissions in terminal patients w/ Hodgkin's & NK/T lymphomas. Procedure completely eliminated cancers. Procedure applicable to all solid & hematologic cancers.

...procedure very gentle, w/o adverse effects. No target toxicity & no cytokine release observed.

...procedure uses no gene incorporation procedure or virusdirected gene incorporation. Manufacturing costs are low at very high manufacturing success rates



Asset incomplete examples (out of 1st 50 R&D drug results)

Pipeline DBs vs. BIO: Main differences for assets/drugs

- Pipeline DB: What it is, lifecycle, milestones
 - What it is: e.g. small molecule, tumor necrosis factor, RhA, under license from, RoA
 - LC/Milestones: e.g. IND for P2 files on date1 for TA1, trial planned, positive/negative data announced, app submitted to SFDA, trial details, conference announcements
- BIO: What it is, how it works, why it's good, objective
 - What it is
 - What are it's selling points: e.g. FiC, how it works in disease pathway, brief desc of studies, but highlighting why results were significant, who else gives it credibility

Asset incomplete examples (out of 1st 50 R&D drug results)

new class of Zn2+ ionophore / anticancer therapeutics (company = Curza)

Pipeline DB: Indexes like TA, MoA, Phase = "Discovery", etc. ... investigating compounds that interfere w/ trace metal homeostasis and promote cell death, for treatment of cancer.

9/16: development ongoing



Asset incomplete examples (out of 1st 50 R&D drug results) (cont'd)

BIO: Indexes like TA, MoA, Phase = "Lead disc./optimization", etc.

...developed heterocyclic core that is selective agent, kills chemo-resistant primary plural effusion cells from refractory breast cancer patients while not affecting untransformed cells. CZ-3-8 is efficacious in-vivo & well tolerated even at high dose. MoA from ability to act as Zn2+ ionophore & cause Zn2+ dyshomeostasis in cancer cells, leading to lysosomal membrane depolarization & necrosis... most metal chelators function either as Zn2+ transporters or Zn2+ chelators. There is a limited # of compounds that demonstrate this type of ionophore activity... all require concentrations > 200 µM to cause cell death. CZ-3-8 first compound that exploits MoA at

therapeutically relevant concentrations. The CZ-3-8 core is readily adaptable to medicinal chemistry.

Meeting trends, segmented by...

At the macro-level, answering questions like what's going on in the industry.

- Requested vs. accepted
- TAs, MoAs, technology, phase, finances, ownership, geography
- Company type (e.g. pharma vs. VC)

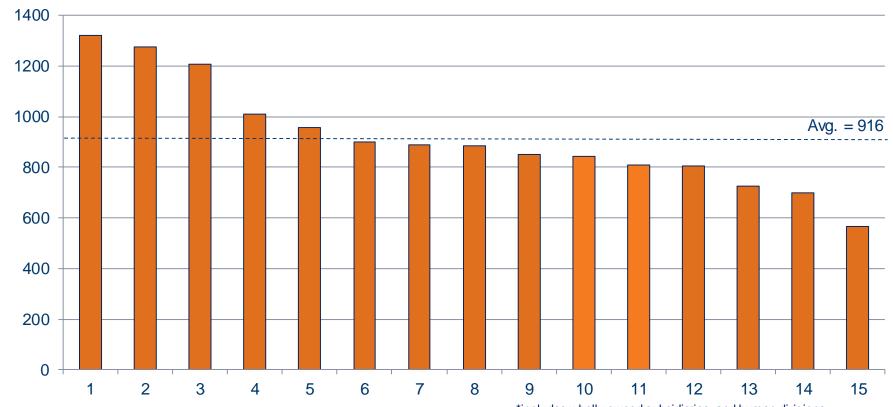
At the micro-level, answering question like what's going on in my company.

- The above analyses, for your company
- How does your company compare to others (aggregated)? # of meetings? Quality of companies proxied by in-licensor meeting count? How many in-licensors vying for your target?



Total Requests Top 15 Pharma by Market Cap, ordered by # Total Requests

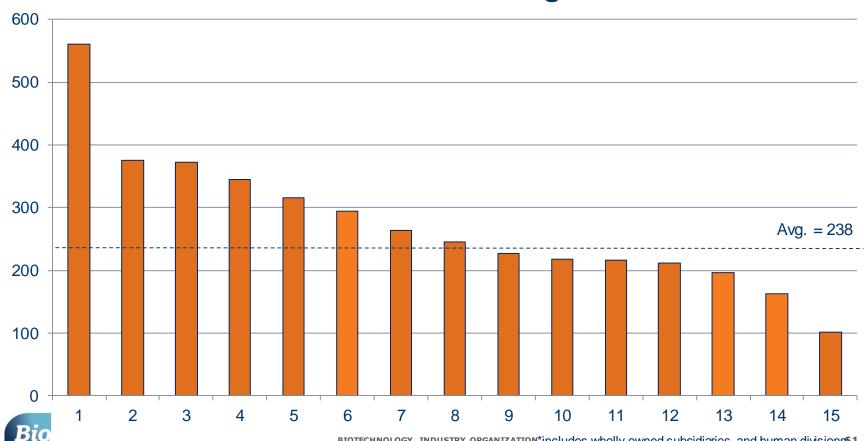
Total Requests





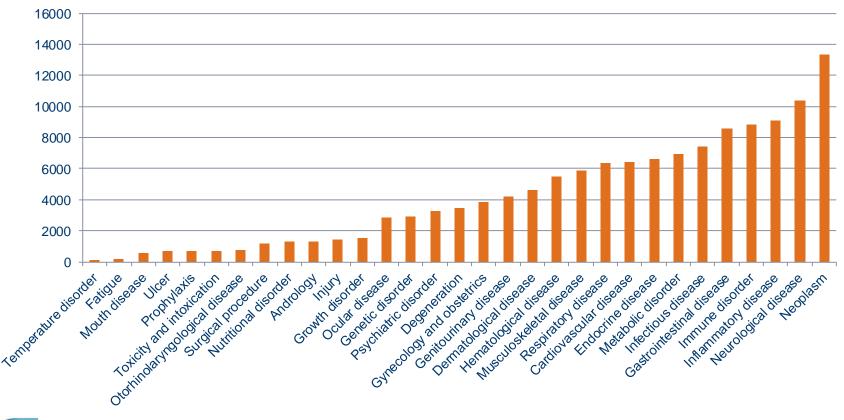
Scheduled Meetings Top 15 Pharma by Market Cap, ordered by # Scheduled Meetings

Scheduled Meetings



Macro ex. Therapeutic area analysis: Prevelance

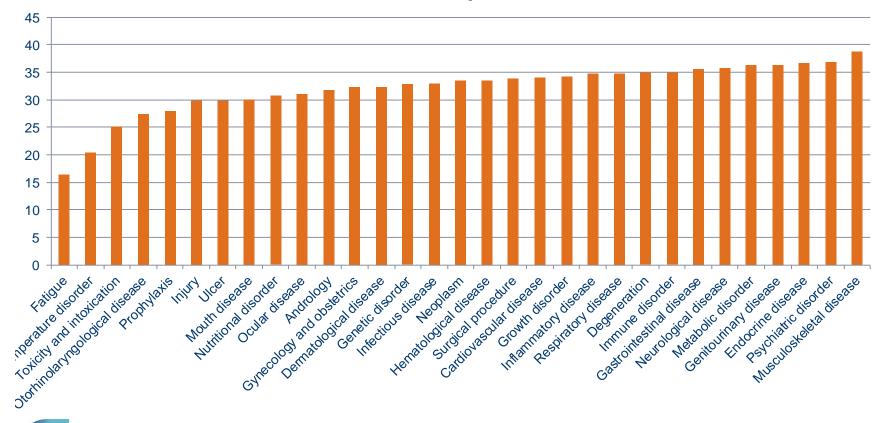
Accepted Requests





Macro ex. Therapeutic area analysis: Success rate

% Accepted





Meeting request templates

- Many companies send variations of the same one or two meeting requests to in-licensors.
- These can be considered templates.
- These sometimes contain detailed information beyond what's available in any pipeline DB or profiles in BIO's system.
- These often contain a better and more meaningful summary, along with clear identification of the lead candidate, than pipeline DB editors provide. Meant to convey most meaningful facets in less than 60 secs. Pipeline DB editors want you to curl up in be with their reports.
- These can be made available with permission from the individual sender (permission secured on a case by case basis by BIO)

Meeting request template example (from 10/16)

Oncternal Therapeutics focuses on embryonically expressed targets which are not expressed on normal cells. We have found that certain cancer cells have developed the ability to hijack these targets and related pathways to both proliferate and migrate.

We currently have **two clinical stage programs in Phase 1b** trials. Our **ROR1 program** is addressing hematological malignancies while our ETS family program addresses Ewing's sarcoma and has additional potential in solid tumors.

Our team has enjoyed past successes at Bavarian Nordic, Candence, Calistoga, Immunogen, Pharmacyclics and Acerta Pharma. Our current financing is targeting approximately \$20MM for our close.



Meeting request template example (from 10/16) (cont'd)

Advantages of meeting request

- Clear summary of the science conveyed vs. pipeline DB: "formed based on research around the ROR1 signaling system and cancer"
- Phase update: ROR1 program is in P 1b; in pipeline DB it's still in "Discovery"
- Is the science or the team more important? Team credentials clearly expressed in the meeting request
- Until 4/17, pipeline DB did not know they were raising another round targeted for \$20M. Pipeline DB picked up \$18.4M funding in 4/17; comparison to \$20MM allows measure of success judgement.



Meeting request template example

Incuron is a privately held clinical-stage oncology company headquartered in Buffalo, NY. We have discovered a family of proprietary small molecules, the Curaxins, with unique multitargeted mechanism of anticancer activity. The **lead Curaxin compound**, **CBL0137**, is in **Phase I clinical development as oral and IV formulations**.

Incuron seeks Round C investment to complete Phase I and run Phase II programs for CBL0137 and provide clinical proof-of-concept.

Key differentiators of Curaxin CBL0137 is an unprecedented MoA that restructures chromatin and strikes simultaneously a set of universal **previously undruggable anticancer targets**: inhibits MYC, NF-kB & HSF-1 and activates p53.



Meeting request template example

Efficacy:

- shown in >30 preclinical in vivo models covering all major cancer types
- crosses blood-brain barrier; efficacious against brain tumor models
- tumor cells fail to develop resistance to Curaxins
- effectively kills tumor stem cells
- synergistic activity with multiple conventional drugs
- intra-arterial (IA) infusion opportunity

Favorable Pharmacology & Safety:

- available in i.v. and oral formulations, t1/2(human) > 10 hrs
- Lacks genotoxicity, regardless of targeting DNA topology

Meeting request template example (from 10/16) (cont'd)

Advantages of meeting request

- A few bits of new information: i.v. form available, looking for round C investment
- The rest is either stated or could be implied from pipeline DB report. So either read...
 - ~ 160 words (45 50 seconds) of the meeting request OR
 - ~1400 words (7 minutes) of pipeline DB report
- Your users want the topline sometimes and the nitty gritty lifecycle details other times. Are they in search and evaluation mode or due diligence mode?
- Pipeline DB is objective, meeting request is subjective (and omits bad news)





Biotechnology Innovation Organization

PART III

BIO One-on-One Partnering:
More than just a partnering tool...

Meeting request template example (from 10/16) (cont'd)



Inova Deals & Alliances (DnA) customers















































































































Future: benchmarking (w/permission, aggregation, anon)





Biotechnology Innovation Organization

PART IV
Information Access

How will BIO Partnering info. be made available?

- Timeline for availability is mid 2018 or sooner
- All information will be compliant with prevailing US and French privacy laws, and made available with permission, aggregated and anonymized as appropriate
- Note BIO terms of use restrict using information for commercial purposes or mass storage in company DBs without permission
- Contact me for more details:

Sougato Das Managing Director, Partnering, BIO sdas@bio.org | 202 345 2258

