

California Institute for
Regenerative Medicine

IP and Industry Sub-Committee
September 23, 2013
San Francisco

Elona Baum
General Counsel / VP Business Development

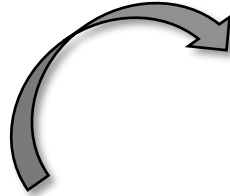


Topics Covered

- Context
- CIRM's Partnering Activities
- Outcomes to Date
- Next Steps



Strategic Goals



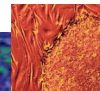
Focus (2011-2016)

- Prioritize projects and investments
- Drive clinical trials for patients to generate preliminary evidence of therapeutic benefit
- Develop partnerships

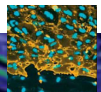
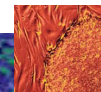
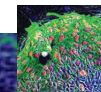
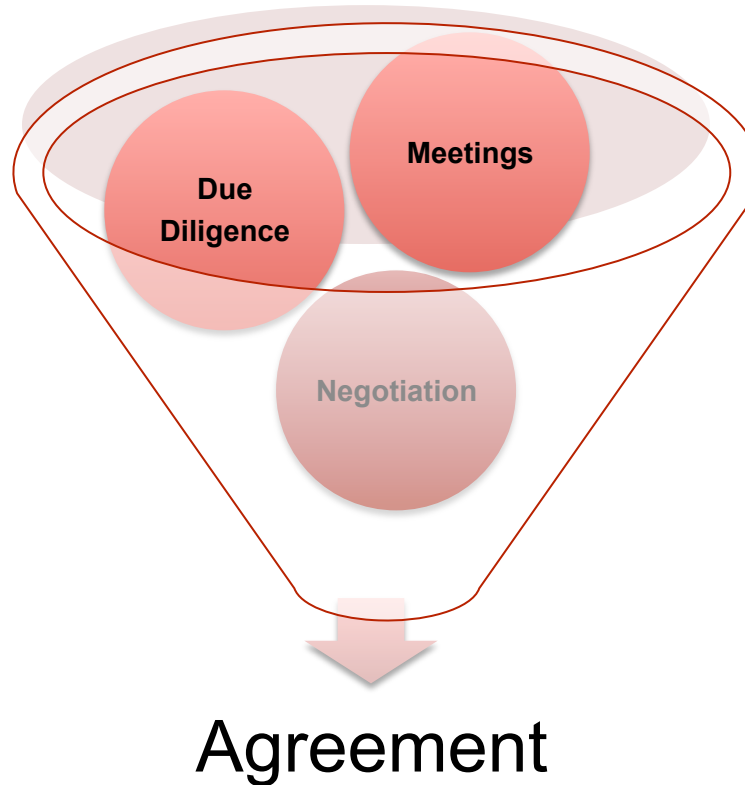
Deliver (2016+)

- Facilitate commercialization of therapies
- Advance therapies to patients
- Enable business model for stem cell-based therapies

CIRM Uses Several Tools to Form Partnerships

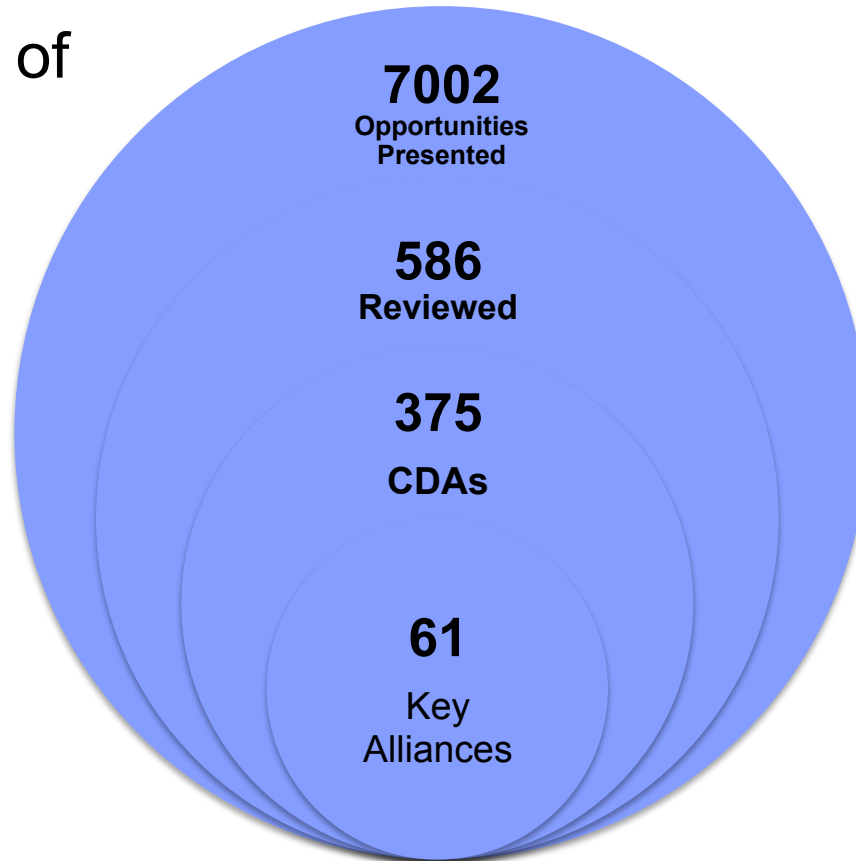


Many Months Required to Generate an Agreement

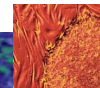


Partnership Formation

Merck 2012:
Perceived Risk of
Project has a
Significant
Impact on
What is
Partnered



(As reported at GTC 6th Annual CNS Partnering Conf.)



CIRMs Development Stage Pipeline

Pre-clinical

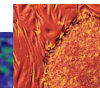
- Conducting IND enabling work, candidate identified
- 24 projects (SP and DT awards)

Ph I

- Calimmune (HIV, DT1 RFA)
- Capricor (Cardiac, DT2 RFA)

Ph II

- No CIRM Funded programs at this stage



Strategic Partnership RFAs: One Tool for Fostering Linkages

- **Approved Scope:** To fund those projects regardless of stage (pre-clinical/clinical) that have demonstrated the ability to attract capital needed to drive programs through clinical trial
 - Provides a vehicle to attract high potential programs with less cycle time
 - Market acts as a proxy for identifying investment interest
 - Creates internal champions within biopharma's
- **Mechanism:** RFA process that permits changes to scope
 - SP1 – SP3 focused on projects close to entry into the clinic
- **Commercial Validation Entry Requirement:**
 - Financial Strength or
 - Agreement with a biopharma having a market cap of at least \$500M



Other Approaches to Foster Linkages with Biopharma/VCs

- Participation at conferences that provide partnering opportunities:
 - JP Morgan
 - BIO International
 - Phacilitate
 - GTC's CNS Partnering Event
 - BIO Europe
 - Stem Cell Meeting on the Mesa



CIRM's Engagement with Industry



121 CIRM Engagements with VCs and Pharmas⁽¹⁾

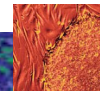


26 VC/Pharma significant outreach to grantees and potential applicants



6 VC/Pharma letters of support in RFAs

(1) As of May 2013; Does not include multiple meetings / conversations with the same party - total represents unique third-party interactions. Meetings include teleconferences and emails.



Illustrative Sales Force Activity Log

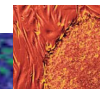


Action	Subject	Location	Date
Edit Del	DT3 Letter of Support & Diligence Update	CIRM Offices	09/09/13
Edit Del	DT3 Letter of Support, Diligence Update & Intro to New Colleague	Company Offices	08/30/13
Edit Del	Update on DT3 Timeline and CIRM Expectations	Telephonic	07/25/13
Edit Del	CALBIO Conference Meeting	San Diego	07/03/13
Edit Del	Update on CIRM Grantee Diligence	Email	05/30/13
Edit Del	General Update on Areas of Interest	CIRM Offices	04/05/13
Edit Del	Introduction to New BD Contact	Email	02/20/13
Edit Del	Phacilitate Meeting	Washington DC	01/28/13
Edit Del	Stem Cell on Mesa Meeting	San Diego	10/15/12



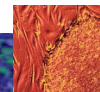
Other Approaches to Foster Linkages with Biopharma/VC's

- Terms of RFAs: Requiring a match to be eligible for an award (or enhanced scoring at GWG or during programmatic review)
- VC “Meet-Up” Day
 - 16 VC’s attended
 - 7 CIRM teams presented
- ARM 1st Annual Regen Med Investor Day
 - 3 CIRM for-profit grantees presented to institutional investors and equity research analysts
- Mentorship Opportunities
 - 2 VC’s are reviewing pitches by CIRM grantees and providing feedback on presentations for SCMOM



Major Biopharma's Comments

- “We will wait for Phase 1 data”
- Concern re heterogeneous mixture of cells
- Two large biopharma's believe that cardio is a good area for regen med., but have not been responsive to CIRM's projects in that area; others looking at hearing and eye loss
- Our company wants to focus on small molecules or antibodies that have endogenous effects at this stage
- PI not interested in partnering at this time



Outcomes: Biopharma and VC Linkages All at Preclinical and Earlier Stage

Biopharma Linkages

6 Letters of support (in connection with DT3, SPs)

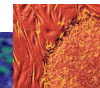
Roche/Versant Company Creation Around CIRM Funded Asset

1 Pharma and 2 VCs Would like to Partner with a CIRM Grantee

Investor/ Company Funding

\$10.6+ M raised by ViaCyte, SP1

\$6.7M Sangamo SP2; Others ++



Early Success

- October 10, 2012 – Roche entered into an exclusive partnership with Versant Ventures and Inception Sciences to create a drug discovery incubator, Inception 3, for the treatment of sensorineural hearing loss
- **Inception 3 will incorporate an innovative technology platform from Stanford University that was previously funded by CIRM**
 - Funding Type: Comprehensive Grant (RC1-00119)
 - Grant Title: Generation of inner ear sensory cells from human ES cells toward a cure for deafness
 - Investigator: Stefan Heller
 - CIRM Funds Committed: \$2.5M
- Versant will provide equity financing and Roche will fund the research based on a series of milestones
- Roche retains an exclusive option to acquire Inception 3 upon a first lead compound reaching the filing stage of an IND

Elsevier Business Intelligence

"The Pink Sheet" DAILY

OCTOBER 10, 2012

Roche Enters Hearing-Loss Space In Risk-Sharing Venture With Inception, Versant

The newly formed Inception 3 will work to bring small molecule candidates for hearing loss, based on technology licensed from Stanford University, to IND-filing stage, at which point Roche will have the option to buy out the program.

A drug-hunting venture borne out of the Bristol-Myers Squibb Co./Amira Pharmaceuticals Inc. buyout in 2011 has resulted in a new opportunity for Roche. Under a novel collaboration structure involving big pharma, venture capital and biotech, Inception Science will create a third company – called Inception 3 Inc. – to discover and develop small molecule drug candidates for sensorineural hearing loss based on technology licensed from Stanford University.

Roche, which will fund Inception 3's work with milestone-based R&D payments, will hold an option to acquire the program upon the filing of the first IND based on the Stanford technology. Inception's backer Versant Ventures, meanwhile, will provide the equity financing for the new company, under an agreement announced Oct. 10.

Inception consists of two current small biotech (Inception 1 Inc. and Inception 2 Inc.) focused on neurology and oncology, founded by former Amira execs after Bristol acquired Amira for \$325 million upfront in July 2011 ("BMS Best On Amira's IPE Drug In \$325M Acquisition" — "The Pink Sheet" DAILY, Jul. 22, 2011). Bristol's focus was on idiopathic pulmonary fibrosis candidate AMB2 and its spin-out much of Amira's remaining intellectual property into new companies called FLAP LLC and Panamera Pharmaceuticals LLC (See Deal). Meanwhile, backed by Versant, former Amira CEO Peppi Prasad, known around the biopharma industry for his "drug-hunting" acumen, established Inception ("After Complex Sale, Amira Scientist And VC Team Up Again For Discovery Start-up" — "The Pink Sheet" DAILY, Jul. 27, 2011).

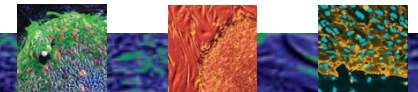
Euca Santarelli, global head of neuroscience at Roche, said his company saw hearing loss as a significant untapped market but one in which its internal R&D personnel was not equipped to lead innovation. Meanwhile, Roche had prior positive experience with Versant from their work together after central nervous system-focused biotech Synosia Therapeutics Inc. was acquired by Biotie Therapies Corp. ("Biotie To Combine With Synosia To Form A 'Global Leader' In CNS Drug Development" — "The Pink Sheet" DAILY, Jan. 11, 2011).

"There is only a limited set of targets and programs that we can [investigate] internally," Santarelli explained in an interview. "We are constantly looking for possibilities to access innovation on the outside in ways that enable us to eventually apply our strength of translational drug development at an appropriate time while at the same time tapping into areas that offer promise and unmet medical need, as well as areas where the mechanism of disease biology is better clarified."

"The reason we didn't go for a straightforward collaboration with academia here ... is that the fact that it brings in a team of drug-hunters with a great track record of discovering drug candidates for intractable targets and then driving those to the IND stage," added Shafiq Virani, head of neuroscience partnering at Roche. "We have the operational component of a drug-development team in addition to a major pharma and exciting best-in-class technology from Stanford."

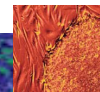
Combined Capabilities Should Produce Rapid Progress
The various parties are not disclosing any financial details about the collaboration nor providing any sense of a timeline to the potential IND filing at FDA. However, Clare Ozawa, chief business officer at Inception and a

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Opportunities for Linkages of Early State Research Assets

- “Early Translation” Programs ET1 and ET2
 - ET1 and ET2 are completed or are in final year
 - While there is no clinical trial data, there may be some level of interest at biopharmas, especially if CIRM will co-fund next phase of work;
 - Approximately 20 programs identified as most promising potentials for biopharma linkages:
 - Disease models
 - Development Candidates



Next Steps:

- Consider establishment of a formal expert mentoring program/ entrepreneur in residence for CIRM grantees
- Consideration of Science Office disease experts
- Additional suggestions?

