



The state stem cell agency

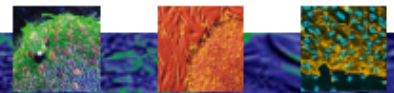
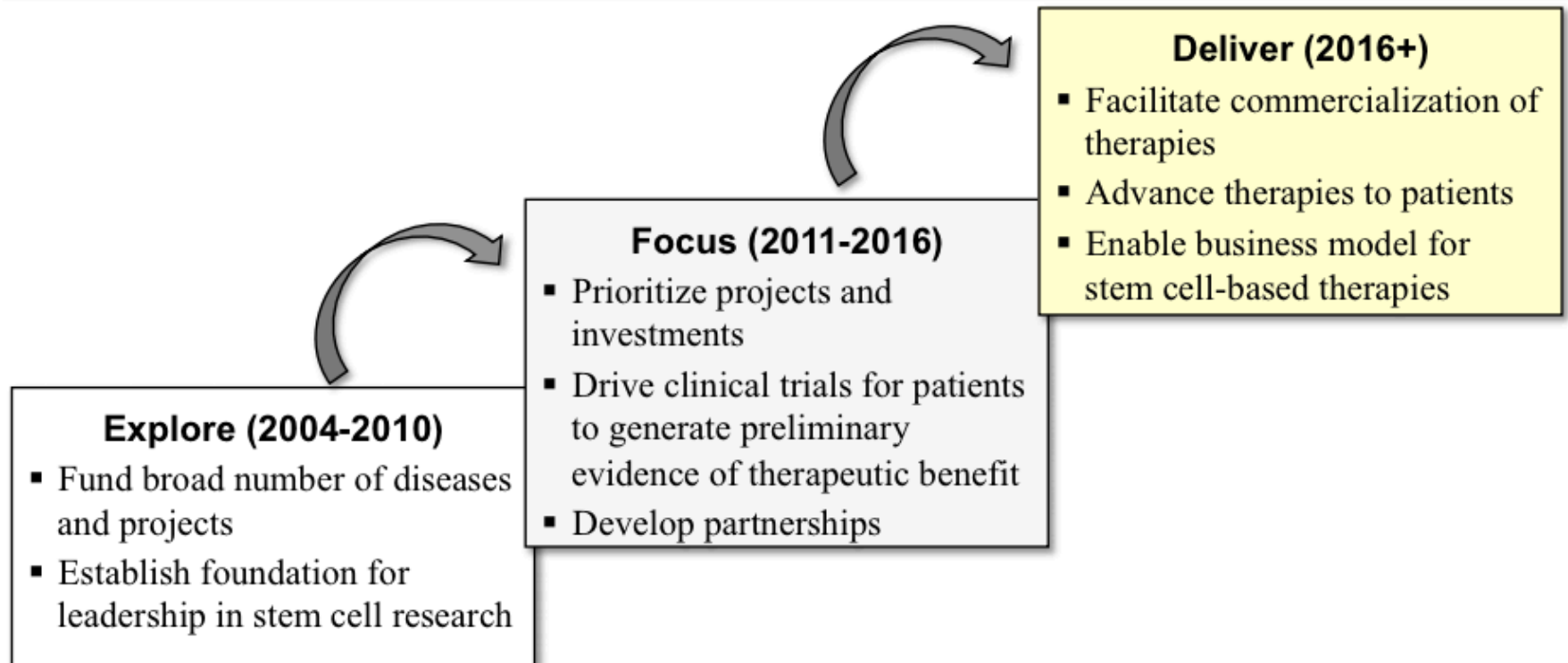
**ICOC Meeting May 23, 2012
Progress to Date:
Industry Engagement &
Commercialization Support
Agenda Item #18**

**By: Elona Baum, General Counsel, VP Business Development &
Neil Littman, Business Development Officer**

The Vision

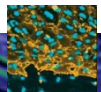
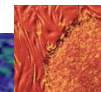
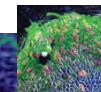
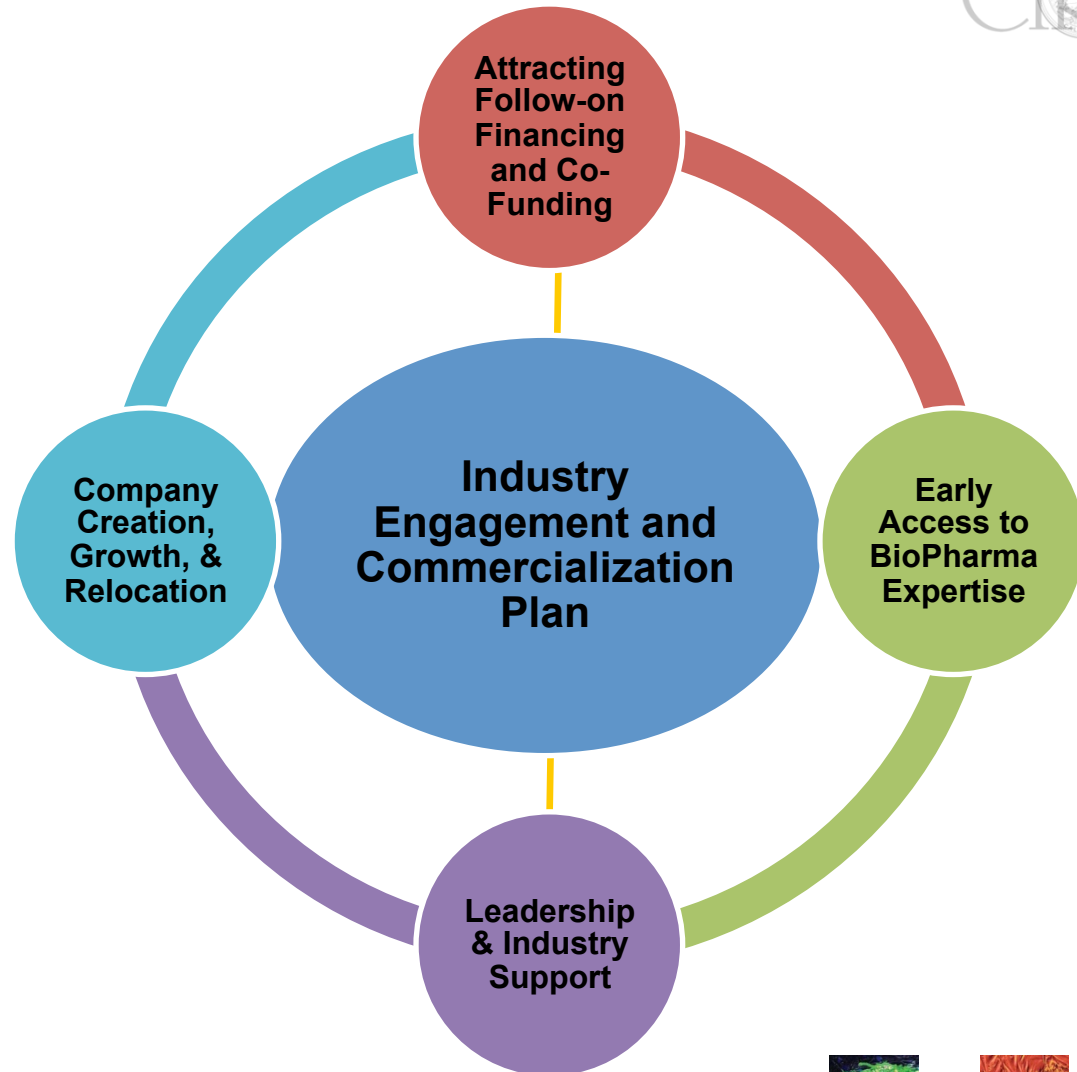
Mission

“To support and advance stem cell research and regenerative medicine under the highest ethical and medical standards for the discovery and development of cures, therapies, diagnostics, and research technologies to relieve human suffering from chronic disease and injury”

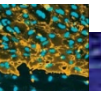
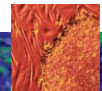
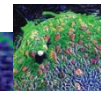


Strategic Objectives of “BD Plan”

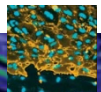
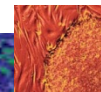
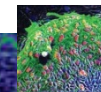
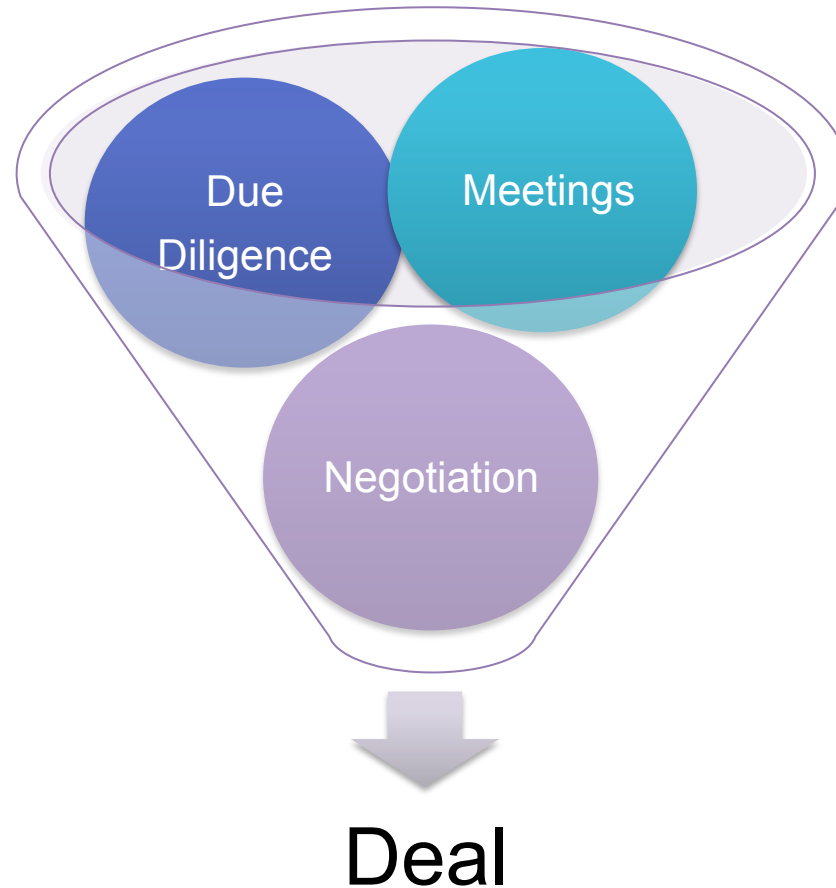
The “Industry Engagement and Commercialization Plan” serves as CIRM’s blueprint for supporting key aspects of Proposition 71 and CIRM’s Scientific Strategic Plan



Follow-on Financing and Co-Funding



Follow-On Financing: Process Takes Time, and it is Early



CIRM as a Catalyst



121 CIRM Engagements with VCs and Pharmas⁽¹⁾



26 VC/Pharma significant outreach to grantees and potential applicants



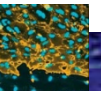
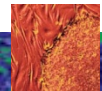
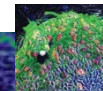
6 VC/Pharma due diligence

- All reports extremely positive with respect to scientific aspects



5 VC/Pharma letters of support in RFAs

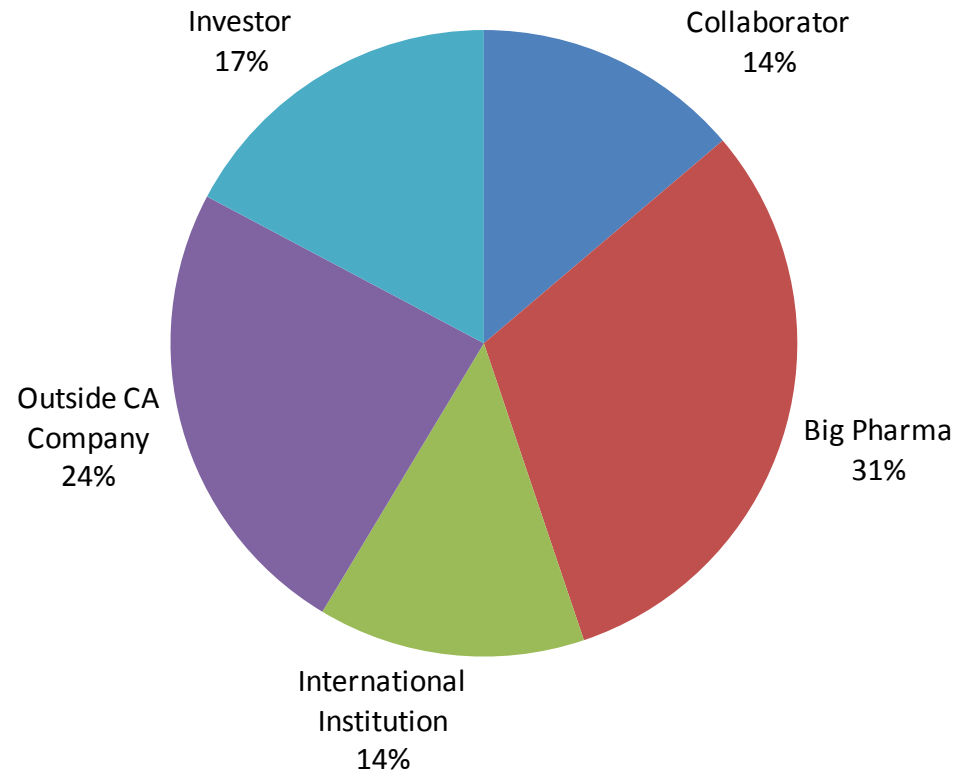
(1) Does not include multiple meetings / conversations with the same party - total represents unique third-party interactions. Meetings include teleconferences and emails.



BIO International Convention Summary

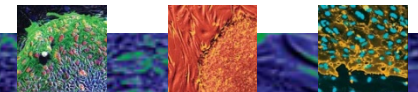


Meeting Summary by Category



- **Total number of one-on-one meetings: 29***

*Includes meetings scheduled during BIO that have subsequently been rescheduled.

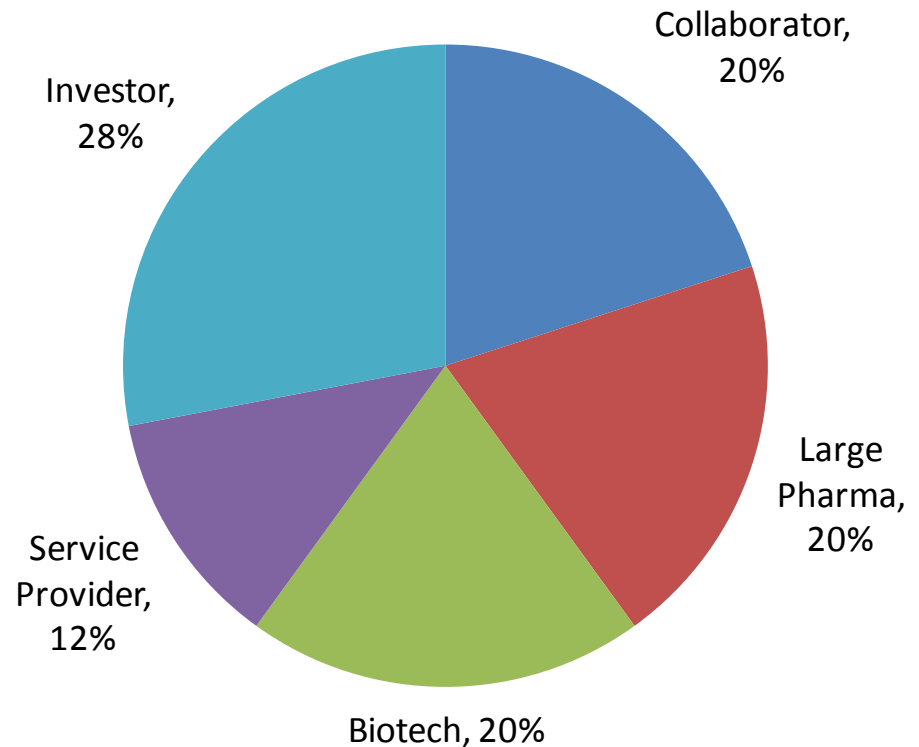


JP Morgan Conference Summary

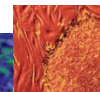
- Majority of meetings focused on highlighting the CIRM portfolio, discussing collaborative funding efforts and continuing to build relationships with:
 - Large Pharma
 - Biotech
 - Investors
 - Collaborators
 - Service providers

- **Total number of meetings: 25***

Meeting Summary by Category



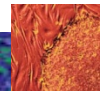
*Includes meetings originally scheduled during JPM that were subsequently rescheduled after the conference.



Several Discussions in Process

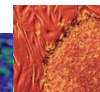


pharma
phacts



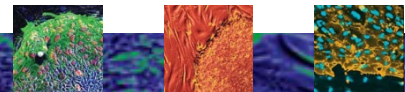
What Have We Learned?

- A number of pharmas prefer not to have their priorities made public – but they have identified areas where they see a RM strategy as advantageous
- One is looking at very early seed funding of companies
- A few are looking at engaging in pre-phase II programs through contributions of in-kind services
- Some looking for clinical POC
- AstraZeneca – RM is a strategic priority in cardio; willing to invest early
- A number of VCs are willing to invest early
 - One DT1 project has been approached by several VCs
 - Another DT1 project is being closely looked at by a VC



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



Attracting Follow-on Funding/ Collaborations



**Follow-on
Funding**



***ToxCast chemical screening program
awarded contract to four US companies to
test up to 10,000 chemicals for potential
toxicity to people and the environment***



Collaboration



VistaGen

***Strategic drug screening
collaboration for candidates for
cardiotoxicity***



VistaGen and Vala Enter Strategic Drug Screening



VistaGen



VALA
SCIENCES

“Our high quality human cardiomyocytes combined with Vala’s high throughput electrophysiological assessment capabilities is yet another example of how we are applying our stem cell technology platform within a strategic ecosystem of complementary leading-edge companies and technologies. **We seek to drive our drug rescue programs forward and generate a pipeline of new, cardiosafe drug candidates.**”

- Shawn K. Singh, JD, VistaGen’s Chief Executive Officer

VistaGen Therapeutics Enters Strategic Drug Screening Collaboration with Vala Sciences

Posted on March 21, 2012 | Comments Off

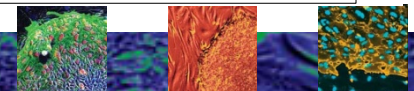
Goal to combine human stem cell-derived cardiomyocytes with novel high-speed kinetic imaging

SOUTH SAN FRANCISCO, CA—(Marketwire – March 21, 2012) – VistaGen Therapeutics, Inc. (OTCBB: VSTA) (OTCQB: VSTA), a biotechnology company applying stem cell technology for drug rescue, and Vala Sciences, Inc., a biotechnology company developing and selling next-generation cell image-based instruments, reagents and analysis software tools, have entered into a strategic collaboration. Their goal is to advance drug safety screening methodologies in the most clinically relevant human in vitro bioassay systems available to researchers today.

Cardiomyocytes are the muscle cells of the heart that provide the force necessary to pump blood throughout the body, and as such are the targets of most of the drug toxicities that directly affect the heart. Many of these drug toxicities result in either arrhythmia (irregular, often fatal, beating of the heart) or reduced ability of the heart to pump the blood necessary to maintain normal health and vigor.

“Our collaboration with Vala directly supports the core drug rescue applications of our Human Clinical Trials in a Test Tube™ platform,” said Shawn K. Singh, JD, VistaGen’s Chief Executive Officer. “Our high quality human cardiomyocytes combined with Vala’s high throughput electrophysiological assessment capabilities is yet another example of how we are applying our stem cell technology platform within a strategic ecosystem of complementary leading-edge companies and technologies. We seek to drive our drug rescue programs forward and generate a pipeline of new, cardiosafe drug candidates.”

Through the collaboration, Vala will use its Kinetic Image Cytometer platform to demonstrate both the suitability and utility of VistaGen’s human pluripotent stem cell derived-cardiomyocytes for screening new drug candidates for potential cardiotoxicity over conventional in vitro screening systems and animal models. VistaGen’s validated human cardiomyocyte-based bioassay system, CardioSafe 3D™, will permit Vala to demonstrate the quality, resolution, applicability and ease of use of its new instrumentation and analysis software to make information-rich, high throughput measurements and generate fundamentally new insights into heart cell drug responses. Accurate, sensitive and reproducible measurement of electrophysiological responses of stem cell-derived cardiomyocytes to new drug candidates is a key element of VistaGen’s CardioSafe 3D™ drug rescue programs. VistaGen’s strategic collaboration with Vala is directed towards this goal.



Co-Funding



\$9.3M

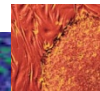
CIRM grant used towards funding Phase 1 Beta Thalassemia trial in conjunction a \$60 million Series D round from private investors – Deerfield, RA Capital, Ramius, ARCH, Third Rock Ventures, TVM Capital, Forbion, Shire



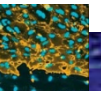
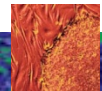
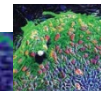
\$3M+



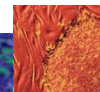
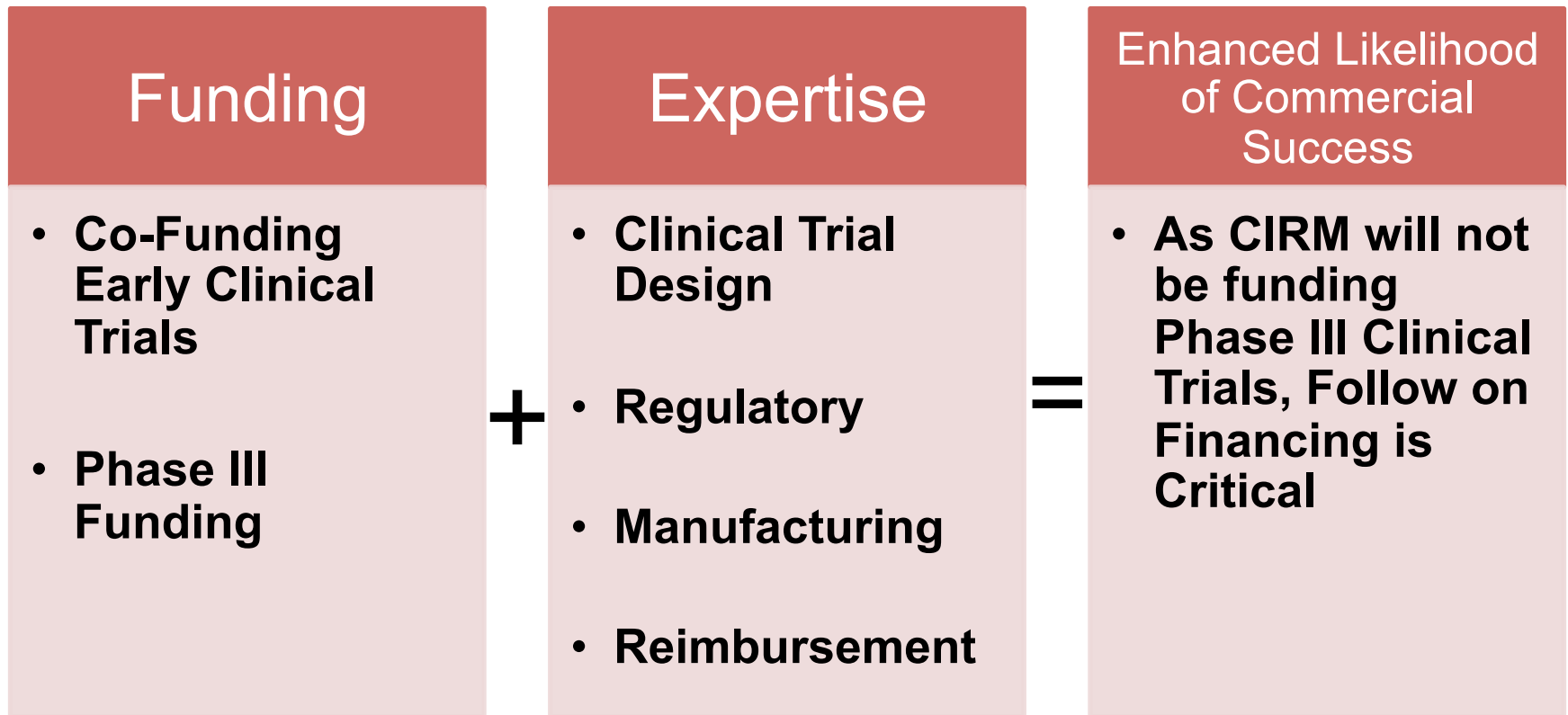
Expectation is this will significantly increase with DT3, SP2 and SP3




Early Engagement of Industry



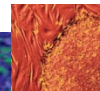
The Benefits Of Engaging Large Pharma And Biotechnology Companies Early



Approaches to Early Access to BioPharma Expertise



Strategic Partnership Program	<ul style="list-style-type: none">• In-Kind Services Count as Part of Match
CIRM Industry Collaborator/Co-Funding Partner	<ul style="list-style-type: none">• Under Exploration
Enhanced Scoring when there is an industry partner	<ul style="list-style-type: none">• Applies to certain RFA's

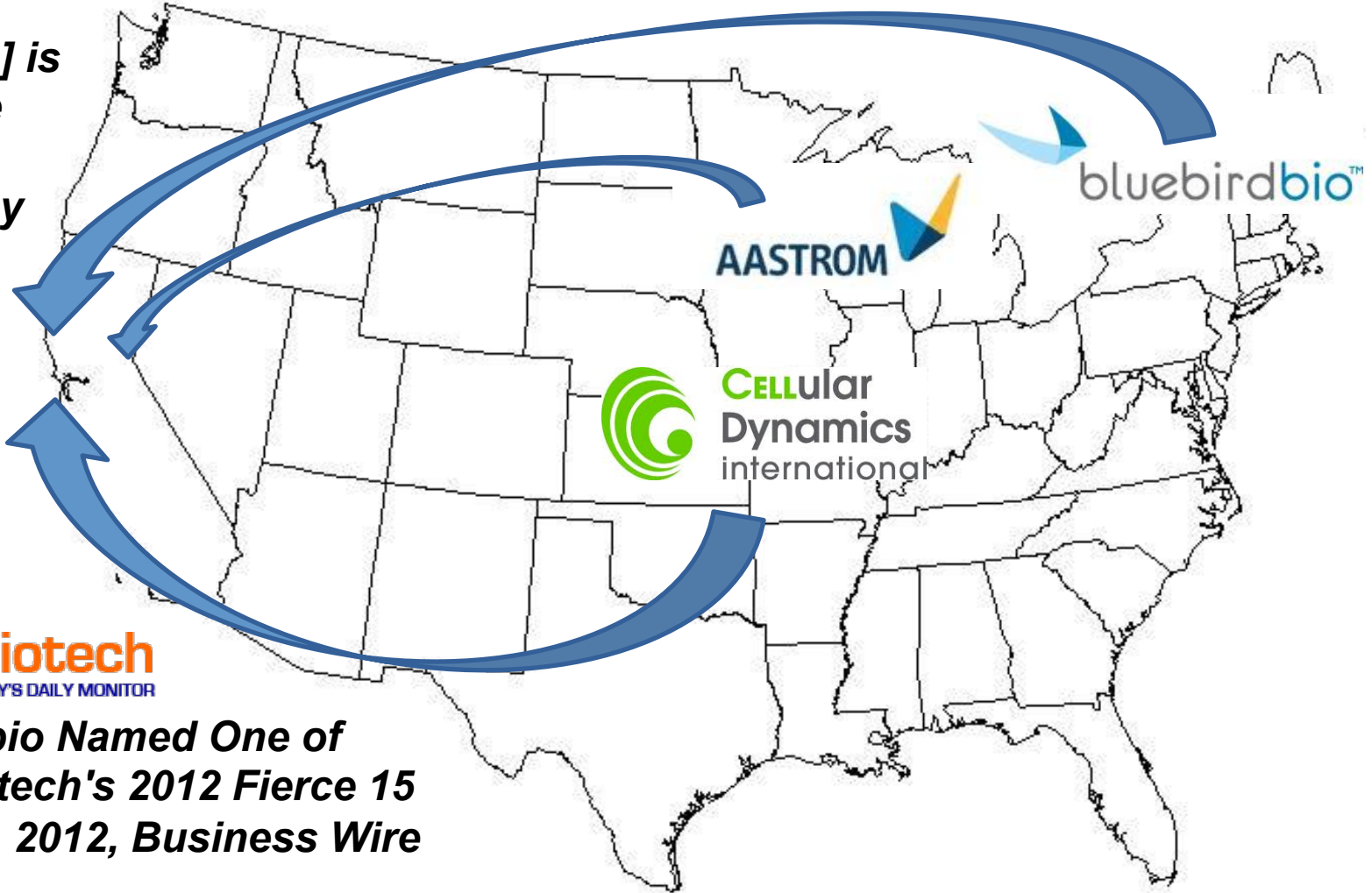


Company Growth, Support and Relocation



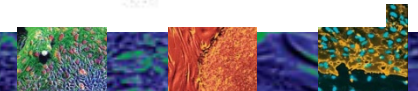
Relocation: Creating Jobs in CA

**“This [CA] is
where the
talent is”
- Company
CEO**



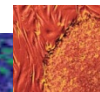
FierceBiotech
THE BIOTECH INDUSTRY'S DAILY MONITOR

**bluebird bio Named One of
FierceBiotech's 2012 Fierce 15
- Sept. 19, 2012, Business Wire**



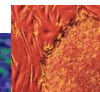
CIRM Funded Spin-Outs

Company	Grant	Technology	Institution/PI
ChemRegen	Seed Grant	Small molecule compounds for stem cell differentiation	Human Biological Research Institute John Cashman
CytoRay	Tools & Technology 1	Wave front sensing technology for use in identifying differentiated and undifferentiated populations of stem cells	UCSC William Sullivan
Didmi	Tools & Technology	CIRM Invention: Substrates for optimized cell culture	Stanford Helen Blau
jCyte	ET-2	Grant title: Human retinal progenitor cells as candidate therapy for retinitis pigmentosa	UCI Henry Klassen
Incerebro	Tools & Technology 2	MicroInjection Catheter System for neurosurgical stem cell delivery	UCSF Daniel Lim
Inception 3	Comprehensive Grant	Generation of inner ear sensory cells from human ES cells toward a cure for deafness	Stanford Stefan Heller



CIRM Funded Spin-Outs

Company	Grant	Technology	Institution
Neurona	Comprehensive Grant	CIRM Funded Invention: "Forebrain enhancers to indentify and select specific types of neural progenitors"	UCSF Arnold Kriegstein
Oceanside Biotechnology	New Faculty	Diagnostic Test for Alzheimers	Western University Doug Ethell
Regenerative Patch Technologies	Disease Team 1	Ophthalmologic hESC derived RPE monolayers on synthetic substrate	USC/UC Santa Barbara Mark Humyan et al
Tolerogen	Seed Grant -- work continued afterwards	Grant Title: "Down-Regulation of Alloreactive Immune Response to hES Cell-Derived Graft Tissues"	UCLA Noriyuki Kasahara
TheraBiologics	Disease Team 1	Allogeneic hNSC line to target tumor, engineered ex vivo to deliver carboxylesterase to locally convert CPT-11 to more potent SN-38	City of Hope Karen Aboody



CIRM-Funded Technology: Incerebro



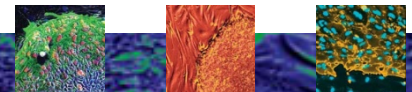
Daniel A. Lim
M.D., Ph.D.

- Assistant Professor in Residence of Neurological Surgery
- Director of Restorative Neurosurgery
- Faculty, Biomedical Sciences Graduate program
- Faculty, Eli and Edythe Broad Center of Regeneration Medicine and Stem Cell Research at UCSF

CIRM Award - \$1,831,723

- Tools and Technologies II Award – RT2-01975
- Development and preclinical testing of new devices for cell transplantation to the brain
- Publication: Stereotact Funct Neurosurg(2013) ***Radially Branched Deployment for More Efficient Cell Transplantation at the Scale of the Human Brain.***
(PubMed: 23343609)

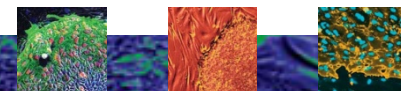
Currently seeking to secure IP from UCSF and to apply for 510(k) approval from the FDA for CIRM-funded device



Review of Industry Awards to Date



For-Profit Grantee	Total Amount	Number of Awards	Type of Award
BioTime, Inc.	\$4,721,706	1	Early Translational I award - \$4,721,706
BlueBird Bio (pending)	\$9,363,335	1	Strategic Partnership- \$9,363,335
Capricor	\$19,782,136	1	Disease Team II- \$19,782,136
Cellular Dynamics International	\$16,000,000	1	hiPSC Derivation - \$16,000,000
Escape Therapeutics, Inc	\$1,453,040	1	Transplantation Immunology - \$1,453,040
Fluidigm Corporation	\$2,693,424	2	Tools & Technology I - \$749,520; Tools & Technology II - \$1,943,904
Gamma Medica-Ideas, Inc.	\$2,478,347	2	Tools & Technology I - \$949,748; Tools & Technology II - \$1,528,599
Geron	\$24,953,095	2	Disease Team Therapy Planning I - \$106,239; Targeted Clinical Development - \$24,846,856
GMR Epigenetics	\$1,452,693	1	Tools & Technologies II - \$1,452,693
iPierian, Inc.	\$7,123,887	2	Early Translational II - \$5,665,887; Basic Biology II - \$1,458,000
OncoMed Pharmaceuticals Inc.	\$65,120	1	Disease Team II Planning - \$65,120
Stem Cells, Inc.	\$19,398,050	2	Disease Team II Planning - \$98,050; Disease Team II (Alzheimer's) - \$19,300,000
Vala Sciences, Inc.	\$906,629	1	Tools & Technology I - \$906,629
Viacyte (formerly Novocell)	\$39,356,426	5	Early Translational I - \$5,405,397; Tools & Technology I - \$827,072; Disease Team Planning - \$48,950; Disease Team I - \$19,999,937; Strategic Partnership- \$10,075,070; Supplementary Funding to DT1 - \$3,000,000
VistaGen Therapeutics, Inc.	\$971,558	1	Tools & Technology I - \$971,558
Wintherix, LLC	\$99,110	1	Disease Team II Planning - \$99,110
Total	\$150,818,556	25	

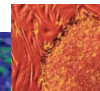


CIRM and QB3

- California Institute for Quantitative Biosciences (QB3) is one of four Governor Gray Davis Institutes for Science and Innovation established to accelerate discovery and innovation
 - Only center that has a focus on the healthcare industry
- CIRM plans to engage in periodic workshops and more informal discussions to inform QB3 Companies of CIRM funding opportunities and requirements



CIRM and QB3 share a mission of advancing and supporting California companies



Leadership and Industry Support



Stem Cell Meeting on the Mesa



3rd Annual Investor and Partnering Forum

October 14 & 15, 2013

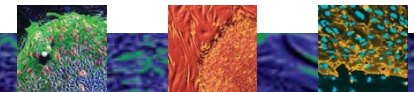
Estancia La Jolla Hotel & Spa | 9700 North
Torrey Pines Road
La Jolla, CA 92037

8th Annual Scientific Symposium

October 16, 2013

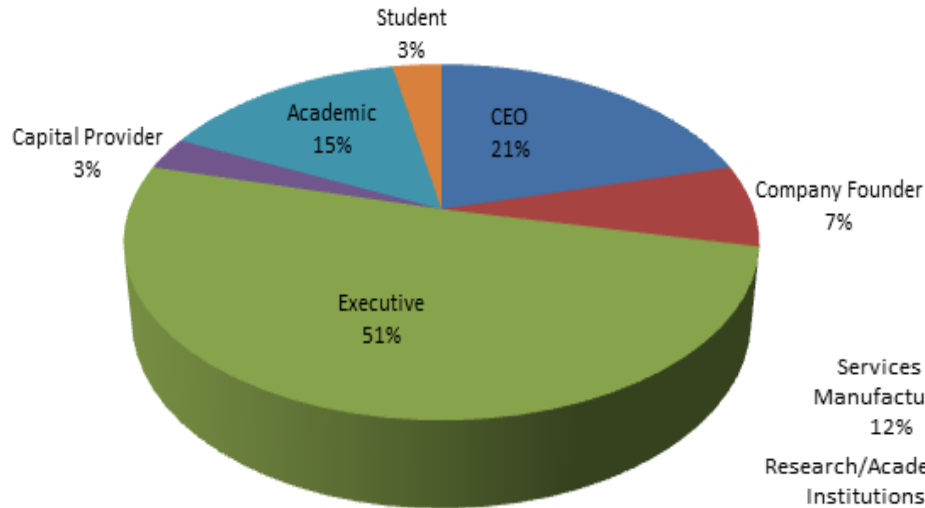
Salk Institute for Biological Studies | 10010
North Torrey Pines Rd
La Jolla, CA 92037

- The Investor and Partnering Forum at the Stem Cell Meeting on the Mesa is the **only partnering meeting organized specifically for the regenerative medicine industry**
- The Alliance for Regenerative Medicine's multi-stakeholder network of companies, investors, research institutes, government agencies, and medical philanthropies provides attendees the opportunity to establish relationships between investors, companies, and entrepreneurial academics to accelerate business development and partnering in the field

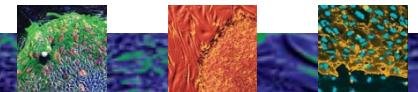
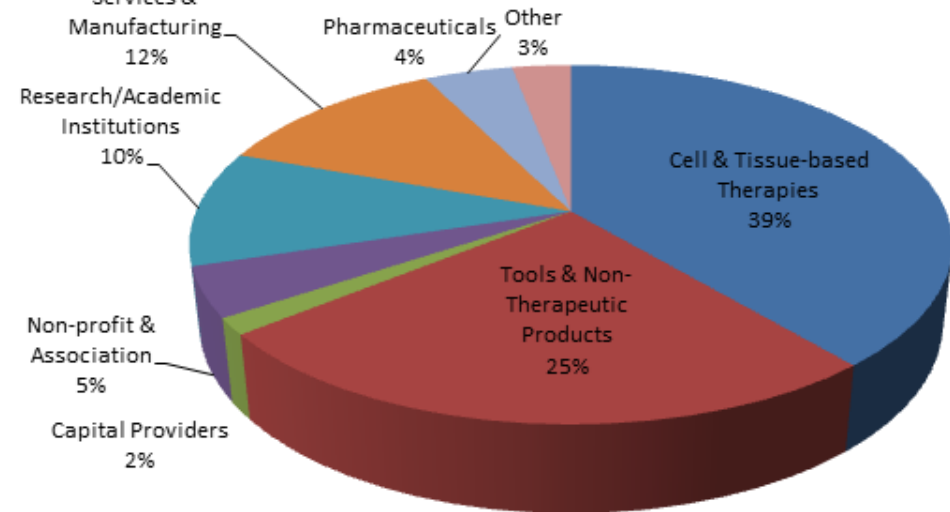


Stem Cell Meeting on the Mesa '12

Attendee Classification



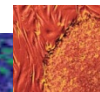
Attendee Industry Focus



Stem Cell Meeting on the Mesa '12



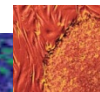
- **Stem Cell Meeting on the Mesa – Investor & Partnering Forum**
- **Total Actual Attendees**
 - 2012 = 290
 - 2011 = 225
 - 29% Increase in Investor & Partnering Forum Attendance
- **Partnering Meetings Scheduled**
 - 2012 = 312 Partnering Meetings
 - 2011 = 199 Partnering Meetings
 - 57% Increase in the Number of Partnering Meetings
- Estimated 61% of the total attendees participated in partnering meetings
- Of those, 88% said they found them to be supportive of their business development objectives
- 95% of them felt they made worthwhile connections
- 73% said it would be okay to follow-up with them in six months to see any development deals/further connections have come out of the meeting then



Leadership & Industry Support



- **Support Provided to Individual CIRM Grantees**
 - Summary valuation and precedent deal summary provided to a DT1 academic team
- **Tools & Tech R&D Roundtable (Summer '13)**
 - Key Tools & Technology Hurdles in Advancing Stem Cell Therapies
 - Topics include: Manufacturing, Assays & Biomarkers, Imaging Technology
- **RegenMed VC Meet-up Day (Summer '13)**
 - Focus on bringing together investors with early stage companies and select CIRM PI's
 - Co-sponsored by CIRM and ARM
- **Reimbursement Webinar (Summer '13)**
 - CIRM hosting a free webinar to be conducted by Holland and Knight



Participation with Industry in Conferences and Events



Phacilitate
CELL & GENE THERAPY
FORUM 2013

De-risking cell & gene therapy development through co-funding: How are novel models for public-private engagement to drive the translation of cell & gene therapy technologies into the clinic actually performing? Defining the opportunities for biotech in each case and delivering the keys to capitalizing upon them

11.35 Case study 1 – NIH

Thomas R. Insel, MD, Director, NIMH; Acting Director, NCATS, NIH

11.55 Questions & discussion

12.00 Case study 2 – CIRM's Strategic Partnership Funding Program

Elona Baum, Esq, General Counsel, California Institute for Regenerative Medicine (CIRM)

12.20 Questions & discussion



THE BUSINESS OF PERSONALIZED MEDICINE

BPM SUMMIT

ADVANCING INDUSTRY INNOVATION
INTO COMMERCIAL SUCCESS



Participation with Industry in Conferences and Events



Financing Alternatives

Funding options for the Life Science Entrepreneur



14 MARCH 2013 | 3:30 PM



PANELISTS

Tracey Mumford | Associate Director, Research Partnerships, The Michael J. Fox Foundation for Parkinson's Research

Kurt Marek | Program Director & SBIR Coordinator, National Heart Lung & Blood Institute (NHLBI)

Neil Littman | Business Development Officer, California Institute for Regenerative Medicine (CIRM)

Lindy Fishburne | Executive Director, Breakout Labs - a project of the Thiel Foundation

Melinda Richter | CEO, Prescience International *moderator*

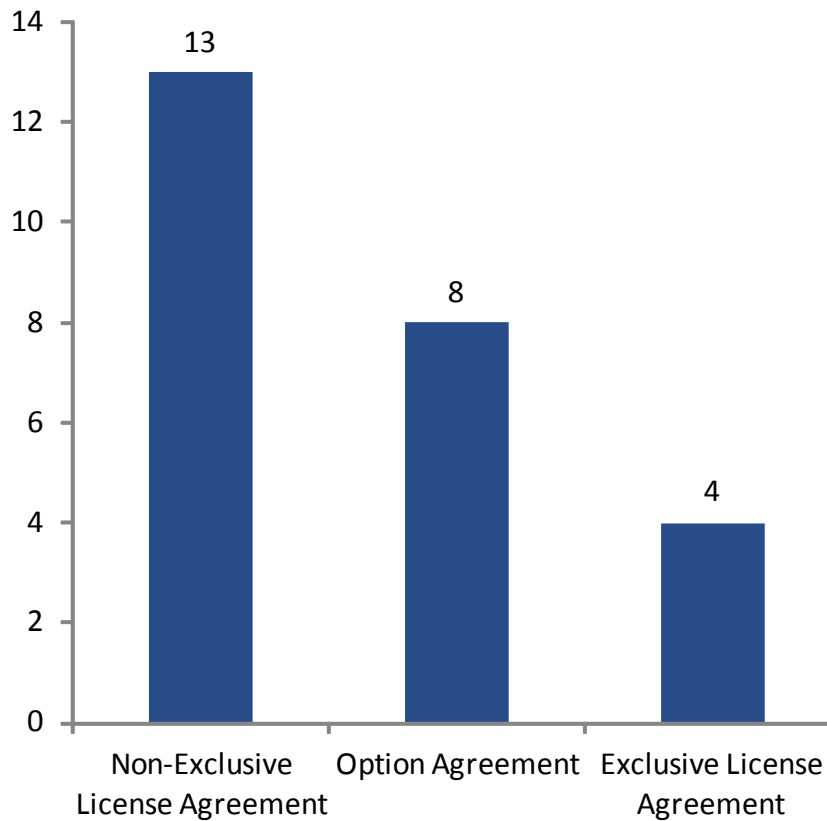
Sponsored by Janssen Labs and Prescience International, CIRM participated in a panel discussing alternative forms of financing



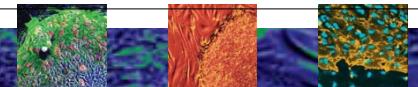
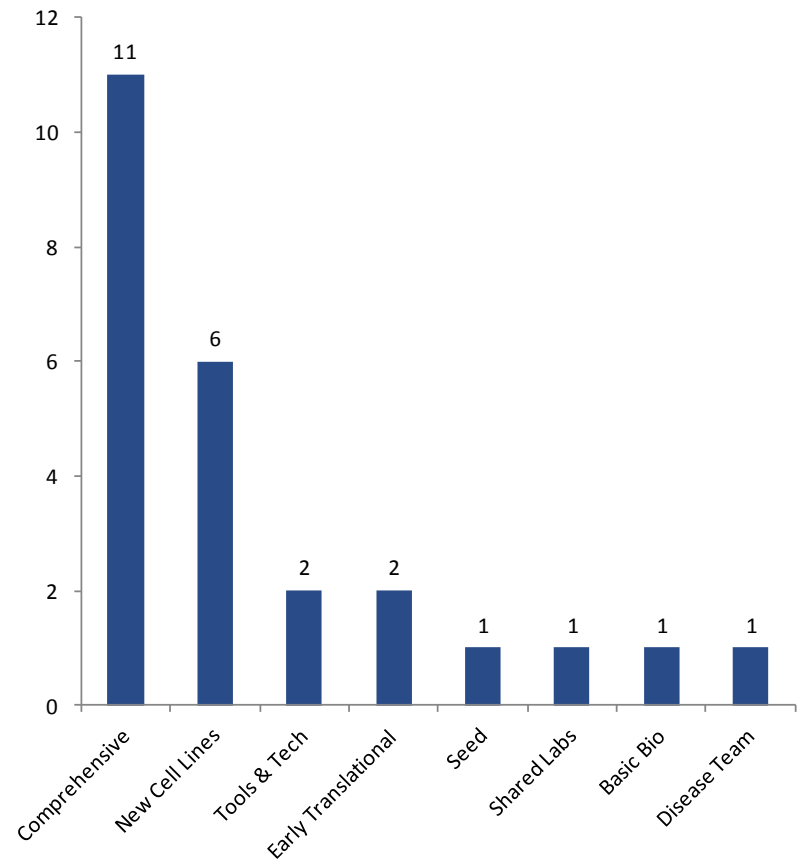
Technology Licensing

(as of 2012 Annual Utilization reports)

By License Type



By Grant Type



Summary

- CIRM's Industry Engagement and Commercialization Plan supports Proposition 71's goal to create an "economic engine" and CIRM's Science Strategic Plan
- Extensive activities underway
 - Linkages have been created
 - Industry support programs being planned
- Already have early successes

