Beyond CIRM 2.0

CALIFORNIA'S STEM CELL AGENCY

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Clinical Portfolio Review

C. Randal Mills, Ph.D.
President and Chief Executive Officer

CIRM's Seamless Pathway

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Program Offerings Per Year

The Clinical Program Has Doubled

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CLINICAL

Pre-IND Meeting or Equivalent → Approved Therapy

Availability
12/Year

All Meritorious Applications

CLINICAL 1 IND ENABLING

For the development of candidates from the Pre-IND meeting stage to filing a successful IND

Amount: $5M
Target 18 months

Prerequisite: Pre-IND Meeting
Next: CLINICAL 2

CLINICAL 2 CLINICAL TRIAL

For the conduct of any phase of a clinical trial

Amount: Up to $20M
Duration: 60 months maximum

Prerequisite: Active IND
Next: CLINICAL 2
As needed

CLINICAL 3 ACCELERATING ACTIVITY

Supplemental funding for opportunistic activities that will accelerate the overall development of an active Clinical 1 or Clinical 2 candidate

Amount: As needed
Duration: As needed

Prerequisite: CLINICAL 1 or CLINICAL 2
Next: None

CIRM Therapeutics Portfolio

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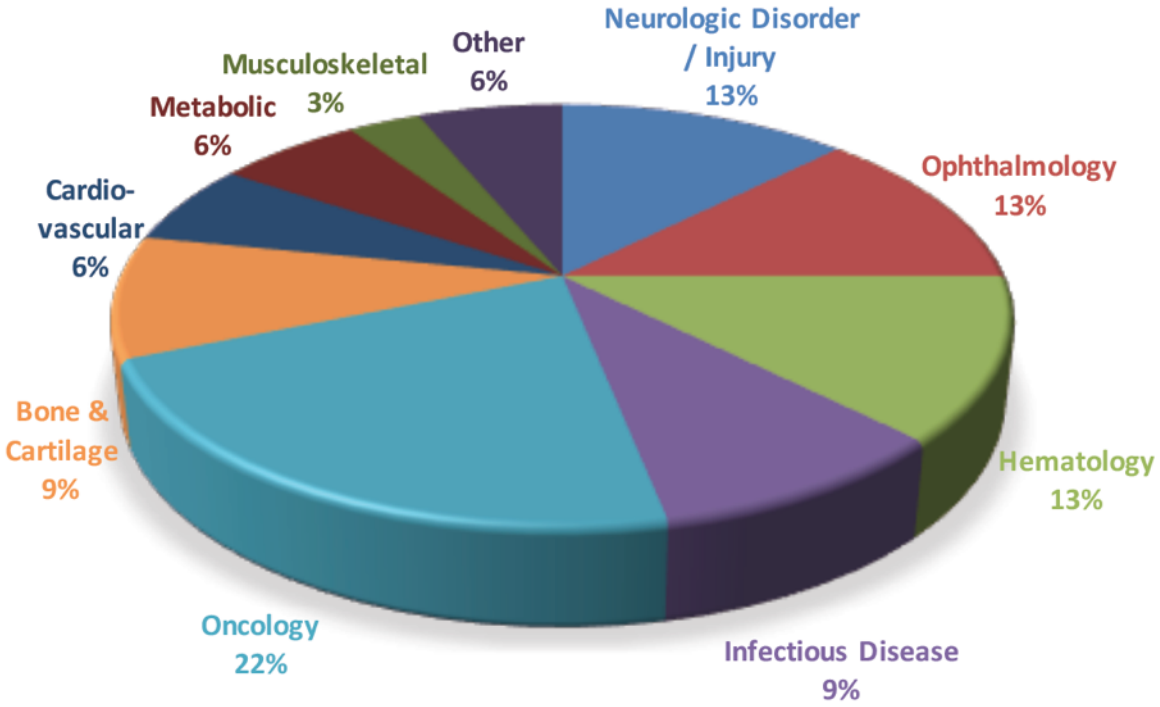
32 individual projects addressing serious conditions



22 Clinical Trials • 10 Pre-IND projects

Clinical Trials

Neurologic and Ophthalmic

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Indication	Investigator / Organization Approach	Phase Status
Spinal Cord Injury	Lebkowski / Asterias hESC-derived oligodendrocyte progenitor cells	Phase 1/2 Enrolling
Retinitis Pigmentosa	Klassen / UC Irvine Neural progenitor cells for injection into eye	Phase 1/2 Enrolling
Age-Related Macular Degeneration	Humayun / USC hESC-derived retinal cells on a synthetic matrix	Phase 1 Enrolling
ALS (Lou Gehrig's Disease)	Svendsen / Cedars-Sinai Neural progenitor cells expressing a protective factor	Phase 1/2 Initiating
Huntington's Disease	Wheelock / UC Davis Monitor natural progression of Huntington's disease	Observational
Spinal Cord Injury	Lebkowski / Geron hESC-derived oligodendrocyte progenitor cells	Phase 1 Closed

Clinical Trials

Organ Systems

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Indication	**Investigator / Organization** Approach	**Phase** Status
Vascular Access	Lawson / Humacyte Engineered blood vessel	Phase 3 Enrolling
Myocardial Infarction	Smith / Capricor Heart-derived progenitor cells	Phase 2 Follow-up
Duchenne Muscular Dystrophy (Cardiac)	Ascheim / Capricor Heart-derived progenitor cells	Phase 2 Follow-up
Type 1 Diabetes	Foyt / Viacyte Encapsulated hESC-derived insulin-producing cells	Phase 1/2 Enrolling
Osteonecrosis	Lane / UC Davis Small molecule recruiting endogenous bone stem cells	Phase 1/2 Enrolling

Clinical Trials

Oncology

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Indication	**Investigator / Organization** Approach	**Phase** Status
Glioblastoma	Gringeri / Immunocellular Autologous cellular vaccine	Phase 3 Enrolling
Solid Tumor	Slamon / UCLA Small molecule inhibiting cancer stem cells	Phase 1 Enrolling
Chronic Lymphocytic Leukemia	Kipps / UCSD Antibody inhibiting cancer stem cells	Phase 1 Enrolling
Acute Myelogenous Leukemia	Weissman / Stanford Antibody inhibiting cancer stem cells	Phase 1 Enrolling
Melanoma	Dillman / Caladrius Autologous cellular vaccine	Phase 3 Closed

Clinical Trials

Hematology

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Indication	**Investigator / Organization** Approach	**Phase** Status
Severe Combined Immunodeficiency	Shizuru / Stanford Chemotherapy-free bone marrow transplant	Phase 1/2 Enrolling
HIV/AIDS	Symonds / Calimmune Stem cell gene therapy to create HIV resistance	Phase 1/2 Follow-up
Chronic Granulomatous Disease	Kohn / UCLA Stem cell gene therapy to fix enzyme in WBC	Phase 1/2 Enrolling
Sickle Cell Disease	Kohn / UCLA Stem cell gene therapy to correct gene defect	Phase 1 Enrolling
HIV/AIDS	Abedi / UC Davis Stem cell gene therapy for AIDS Lymphoma	Phase 1 Enrolling
HIV/AIDS	Zaia / City of Hope Stem cell gene editing to create HIV resistance	Phase 1 Enrolling

Featured Program
Retinitis Pigmentosa

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INVESTIGATOR
Henry Klassen, MD, PhD

INSTITUTION
U.C. Irvine

AWARD
$17 M to conduct a Ph 1/2 trial

APPROACH
Direct injection of neural progenitor cells into the damaged retina to save and regrow rods and cones

Rosie NAME

- Mother of twin girls and son
- Diagnosed at age 26
- Blind in both eyes at treatment
- Left eye injected September of 2015
- Visual acuity has improved to enable reading

Featured Program
Spinal Cord Injury

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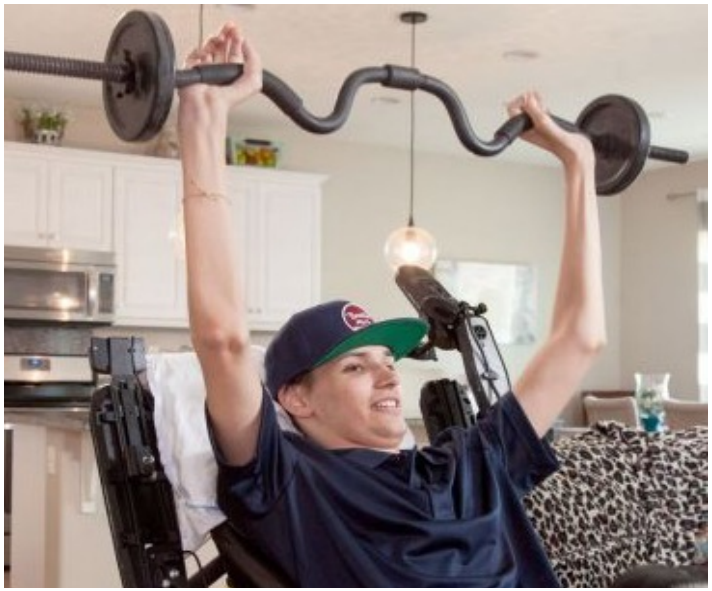
INVESTIGATOR
Jane Lebkowski, PhD

INSTITUTION
Asterias Biotherapeutics

AWARD
$14.3 M to conduct a Ph 1/2 trial

APPROACH
Direct injection of hESC-derived oligodendrocyte progenitor cells into patients with neurologically complete cervical (C5-7) SCI.



Kris Boesen

- Injured in a car crash March 6, 2016
- Completely paralyzed from the neck down
- Treated with 10M cells in April, 2016
- Regained two levels of motor and sensory function

Featured Program
Chronic Granulomatous Disease

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INVESTIGATOR
Donald B. Kohn, MD

INSTITUTION
UCLA

AWARD
$7 M to conduct a Ph 1/2 trial

APPROACH
Autologous bone marrow stem cell gene therapy to replace the defective enzyme in blood cells in patients with CGD.

Brenden Whittaker

- Diagnosed at age one
- Defective gene leads to weak immune system unable to fight infections
- Lost portions of lung and liver
- December 2015 got transplant of his own gene-modified blood stem cells

MISSION

CIRM's Mission

Accelerate stem cell treatments to patients with unmet medical needs.