



SOMETHING  
BETTER  
THAN HOPE

Right now.

**Gil Sambrano, Ph.D.**

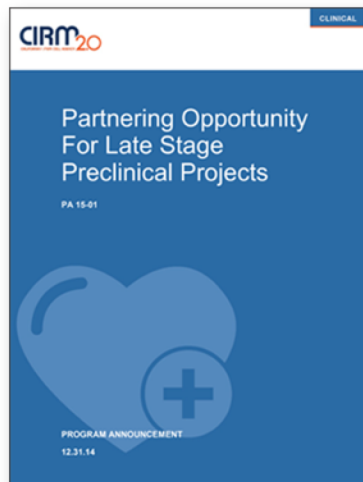
Vice President, Portfolio Development  
& Review  
California Institute for Regenerative  
Medicine

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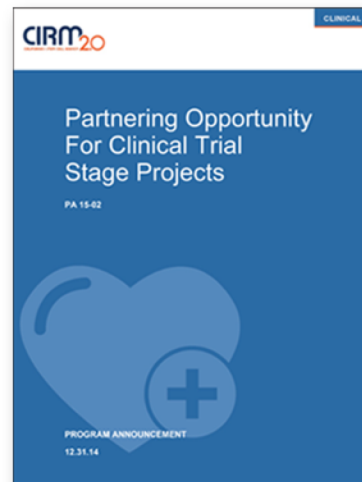


Every Moment Counts. **Don't Stop Now.**

# Clinical Stage Programs



**CLIN 1**



**CLIN 2**



**CLIN 3**

# Scoring System for Clinical Applications

- **Score of “1”**

*Exceptional merit and warrants funding.*

- **Score of “2”**

*Needs improvement and does not warrant funding at this time but could be resubmitted to address areas for improvement.*

- **Score of “3”**

*Sufficiently flawed that it does not warrant funding and the same project should not be resubmitted **for at least 6 months.***

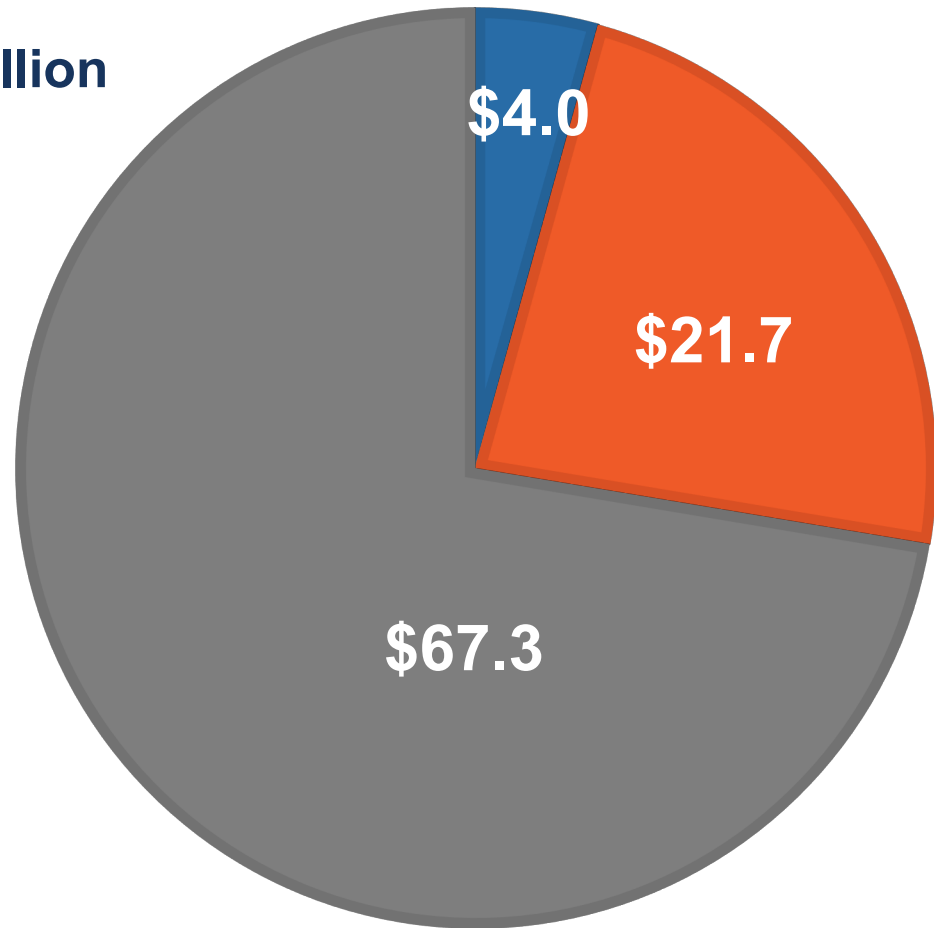
Applications are scored by all scientific members of the GWG with no conflict.

# 2019 Clinical Budget Status

**Annual Allocation: \$93 million**

- Amount Requested Today
- Approved Awards
- Unused Balance

Amounts are shown in millions



# 2019 Clinical Award Targets

## CLIN2 Clinical Trials



## CLIN1 Late Stage Preclinical



 Approved Award       Awaiting Today's Approval

# CLIN1-11256: Project Summary

<b>Therapy</b>	Liposomal formulation of recombinant human WNT3A
<b>Indication</b>	Degenerative Spondylolisthesis
<b>Goal</b>	Completion of IND-enabling studies and GMP manufacturing, IND filing.
<b>Funds Requested</b>	\$3,994,246 (\$998,562 Co-funding)

Maximum funds allowable for this category: \$6,000,000

# CLIN1-11256: Background Information

**Clinical Background:** DS is caused by degeneration of spine tissue resulting in a lumbar vertebrae slipping onto the one below it. Common DS symptoms include lower back and leg pain. DS affects older patients and is more common in women.

Annually, over 90,000 spinal fusion surgeries are performed in patients with symptomatic DS in the US (Spine Journal). Spinal fusion is achieved with implantation of autologous bone graft or graft substitutes such as BMP2.

**Value Proposition of Proposed Therapy:** The osteogenic properties of autologous bone grafts decline with age and use of BMP2 is associated with various local and systemic risks. The WNT3A therapy has potential as a safe anabolic agent to improve osteogenic activity of autologous bone grafts in orthopedic procedures where such grafts are used.

**Why a stem cell project:** The therapy targets endogenous osteoprogenitor cells.

# CLIN1-11256: Related CIRM Portfolio Projects

Application/ Award	Project Stage	Project End Date	Indication	Candidate	Mechanism of Action
Current Application	IND	N/A	Degenerative spondylolisthesis	Liposomal formation of recombinant WNT3A	Anabolic agent activates osteoprogenitors to differentiate and generate bone
CLIN2	Phase 1/2	05/31/20	Osteonecrosis	LLP2A-Alendronate	Anabolic agent activates osteogenic differentiation and homing of MSC to injured bone and promotes revascularization



# CLIN1-11256: Previous CIRM Funding

Applicant has received previous funding from CIRM for discovery and development of the proposed therapy.

Project Stage	Project Outcome	Project Duration	Award Amount	Milestones
Translational (TRAN1)	Pre-IND Meeting	03/01/17 - 11/30/18	\$2,088,780	<b>OM1:</b> Scalable Manufacturing Process ( <b>On Time</b> )
				<b>OM2:</b> Manufacturing, pre-IND meeting ( <b>On Time</b> )
Candidate Discovery (TR1)	POC with Candidate	09/01/09 - 10/31/18	\$6,464,126	14 milestones: 12 achieved, 2 partially achieved. Project progressed to TRAN1

# CLIN1-11256: GWG Review

**GWG Recommendation:** Exceptional merit and warrants funding

Score	GWG Votes
1	12
2	3
3	0

**CIRM Team Recommendation:** Fund (concur with GWG recommendation)

**Award Amount:** \$3,994,246\*

\*Final award shall not exceed this amount and may be reduced contingent on CIRM's final assessment of allowable costs and activities.