CIRM
CALIFORNIA'S STEM CELL AGENCY

Clinical Program
GWG Recommendations

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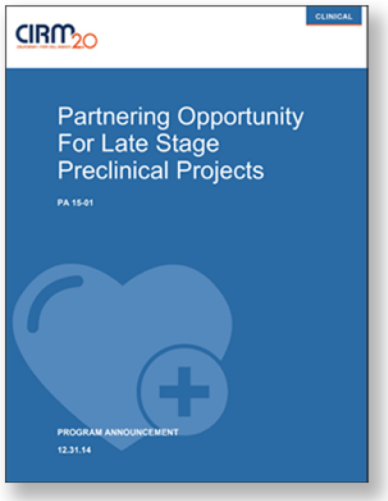
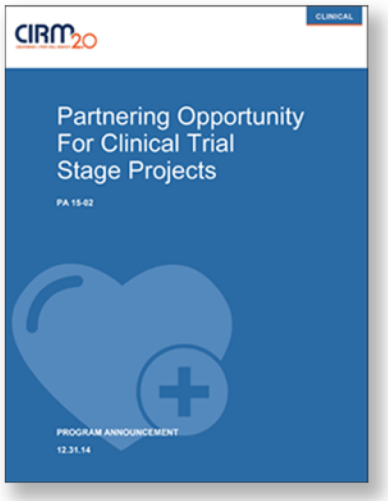
TRANSFORMING medicine lives futures

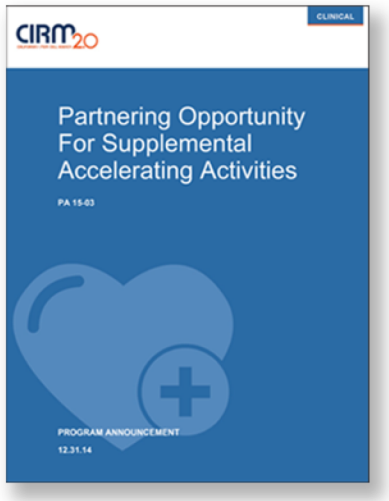
October 18, 2018

Clinical Stage Programs

CLINICAL STAGE







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.

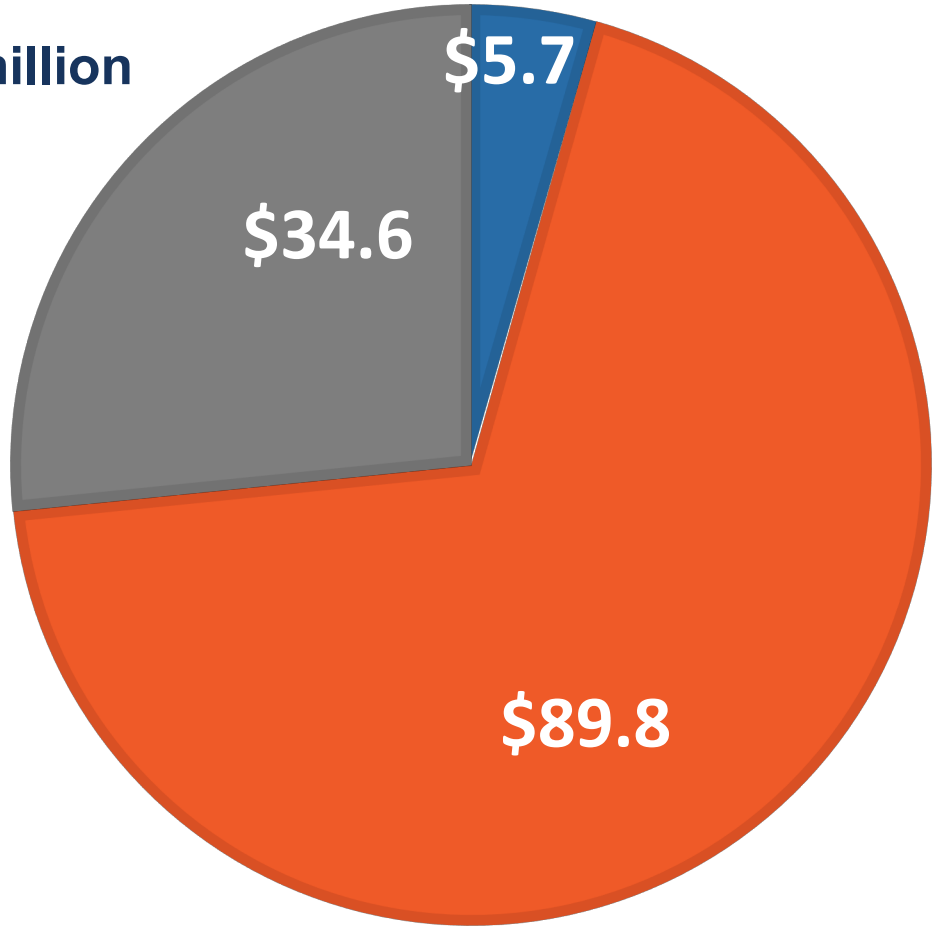
- **Score of "3"**

  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.

2018 Clinical Budget Status
End of September

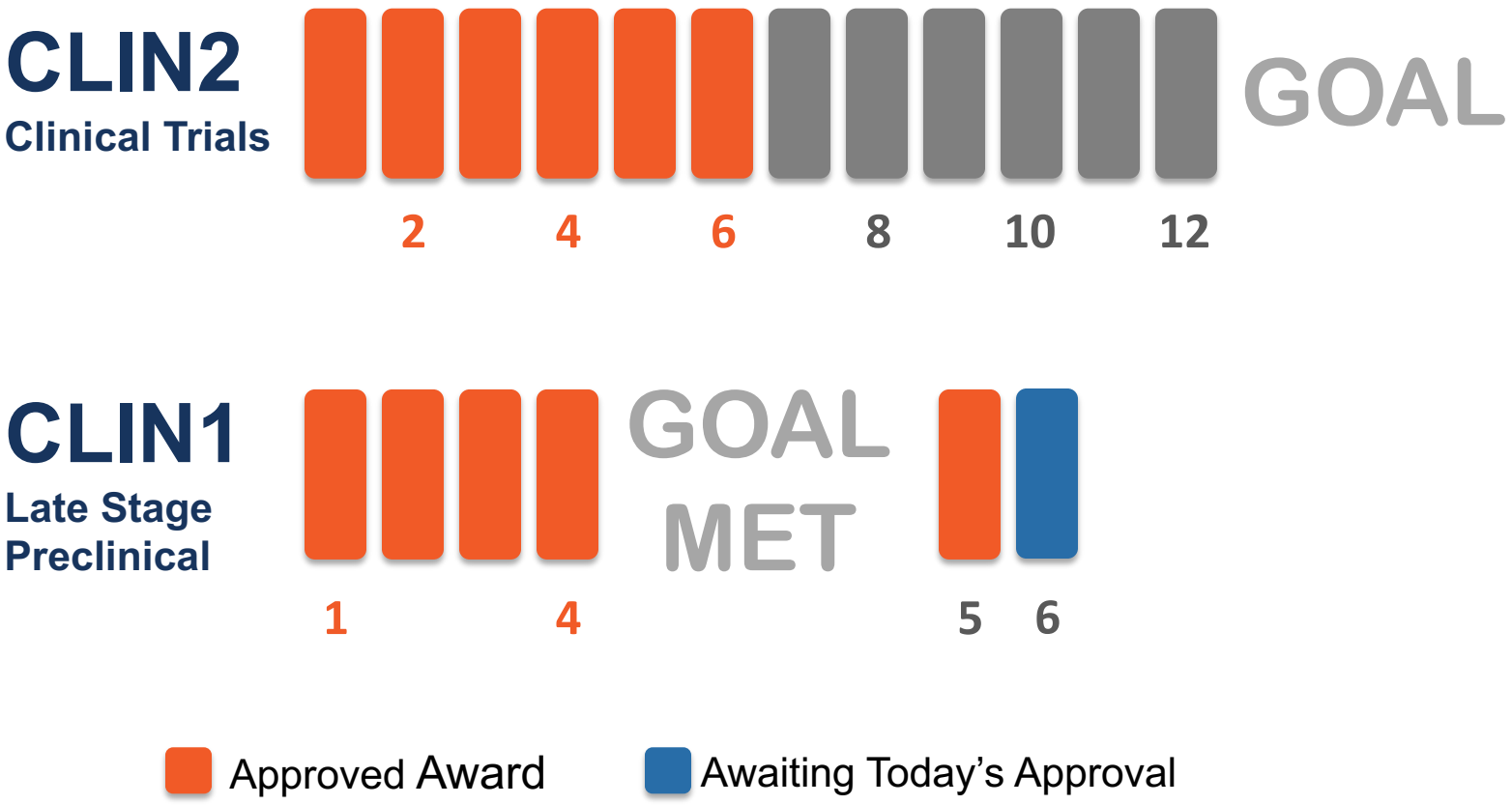
Annual Allocation: $130 million



- Amount Requested Today
- Approved Awards
- Unused Balance

Amounts are shown in millions

2018 Clinical Award Targets



CLIN1-11404: Late-Stage Preclinical Studies of Therapy for Myelomeningocele

Project Summary

Therapy	Placenta-derived mesenchymal stem cells seeded on collagen extracellular matrix
Indication	Prenatally diagnosed myelomeningocele (spina bifida)
Goal	Product manufacturing, conduct preclinical safety and efficacy studies, prepare and submit IND
Funds Requested	$5,666,077 ($0 Co-funding)

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

CLIN1-11404: Late-Stage Preclinical Studies of Therapy for Myelomeningocele

Potential impact: About 1500 babies are born with spina bifida in the US annually (CDC). Spina bifida disproportionately affects children of Hispanic or Latino descent. Myelomeningocele is the most severe form resulting in paralysis below the spinal opening along with Chiari II malformation and hydrocephalus.

Value Proposition: Until recently, the most common treatment was postnatal surgical closure to prevent infection. The NIH sponsored MOMS study demonstrated that fetal surgical closure reduced need for shunting and improved motor outcome. The proposed neuroprotective stem cell therapy could augment fetal surgery by further improving motor outcome and reducing disability.

Why a stem cell project: This is a cell therapy composed of placenta-derived mesenchymal stem cells.

Related CIRM Portfolio Projects

{There are currently no active clinical-stage spina bifida projects being supported by CIRM funding.}

Previous CIRM Funding

Project Stage	Project Outcome	Project End Date
Translational	Conducted Pre-IND Meeting	08/31/2018

CLIN1-11404: Late-Stage Preclinical Studies of Therapy for Myelomeningocele

GWG Recommendation: Exceptional merit and warrants funding

Score	GWG Votes
1	15
2	0
3	0

CIRM Team Recommendation: Fund (concur with GWG recommendation)

Award Amount: $5,666,077*

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