

BETH C. DRAIN, CA CSR NO. 7152

BEFORE THE
INDEPENDENT CITIZENS' OVERSIGHT COMMITTEE
AND THE APPLICATION REVIEW SUBCOMMITTEE
TO THE
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE
ORGANIZED PURSUANT TO THE
CALIFORNIA STEM CELL RESEARCH AND CURES ACT
REGULAR MEETING

LOCATION: AS INDICATED ON THE AGENDA

DATE: NOVEMBER 15, 2018
11 A.M.

REPORTER: BETH C. DRAIN, CSR
CA CSR. NO. 7152

FILE NO.: 2018-13A

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I N D E X

ITEM DESCRIPTION	PAGE NO.
OPEN SESSION:	
1. CALL TO ORDER.	3
2. ROLL CALL.	3
3. CONSIDERATION OF APPLICATIONS SUBMITTED IN RESPONSE TO CLINICAL TRIAL STAGE PROJECTS	13
4. CONSIDERATION OF AMENDMENTS TO THE CONCEPT PLANS FOR TRANSLATION AND CLINICAL STAGE RESEARCH PROJECTS.	5
CLOSED SESSION	NONE
5. DISCUSSION OF CONFIDENTIAL INTELLECTUAL PROPERTY OR WORK PRODUCT, PREPUBLICATION DATA, FINANCIAL INFORMATION, CONFIDENTIAL SCIENTIFIC RESEARCH OR DATA, AND OTHER PROPRIETARY INFORMATION RELATING TO APPLICATIONS SUBMITTED IN RESPONSE TO AGENDA ITEMS "4" AND "5" ABOVE. (HEALTH & SAFETY CODE 125290.30(F) (3) (B) AND (C)).	
6. PUBLIC COMMENT.	NONE
7. ADJOURNMENT.	35

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NOVEMBER 15, 2018; 11 A.M.

CHAIRMAN THOMAS: I'D LIKE TO CALL THE MEETING TO ORDER FOR THE NOVEMBER ICOC AND APPLICATION REVIEW SUBCOMMITTEE MEETING. MARIA, WILL YOU PLEASE CALL THE ROLL.

MS. BONNEVILLE: GEORGE BLUMENTHAL. LINDA BOXER.

DR. BOXER: HERE.

MS. BONNEVILLE: DAVID BRENNER. LARS BERGLUND.

DR. BERGLUND: HERE.

MS. BONNEVILLE: DEBORAH DEAS.

DR. DEAS: HERE.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: HERE.

MS. BONNEVILLE: JUDY GASSON.

DR. GASSON: HERE.

MS. BONNEVILLE: BERT LUBIN.

DR. LUBIN: HERE.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: HERE.

MS. BONNEVILLE: STEPHEN JUELSGAARD.

MR. JUELSGAARD: HERE.

MS. BONNEVILLE: SHERRY LANSING.

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1 LINDA MALKAS.
2 DR. MALKAS: HERE.
3 MS. BONNEVILLE: DAVE MARTIN.
4 DR. MARTIN: HERE.
5 MS. BONNEVILLE: LEON FINE.
6 DR. FINE: HERE.
7 MS. BONNEVILLE: LAUREN MILLER. ADRIANA
8 PADILLA. JOE PANETTA.
9 MR. PANETTA: HERE.
10 MS. BONNEVILLE: FRANCISCO PRIETO.
11 DR. PRIETO: HERE.
12 MS. BONNEVILLE: ROBERT QUINT.
13 DR. QUINT: HERE.
14 MS. BONNEVILLE: AL ROWLETT. SUZANNE
15 SANDMEYER. JEFF SHEEHY.
16 MR. SHEEHY: HERE.
17 MS. BONNEVILLE: OSWALD STEWARD.
18 DR. STEWARD: HERE.
19 MS. BONNEVILLE: JONATHAN THOMAS.
20 CHAIRMAN THOMAS: HERE.
21 MS. BONNEVILLE: ART TORRES.
22 MR. TORRES: HERE.
23 MS. BONNEVILLE: KRISTINA VUORI.
24 DR. VUORI: HERE.
25 MS. BONNEVILLE: DIANE WINOKUR.

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1 MS. WINOKUR: HERE.

2 MS. BONNEVILLE: WE HAVE A QUORUM, AND I
3 WANT TO THANK EVERYONE FOR MAKING TIME FOR THIS AT
4 SUCH SHORT NOTICE. SO THANK YOU.

5 CHAIRMAN THOMAS: THANK YOU, MARIA.

6 WE'RE GOING TO START WITH ITEM NO. 4,
7 WHICH IS CONSIDERATION OF AMENDMENTS TO THE CONCEPT
8 PLANS FOR TRANSLATION AND CLINICAL STAGE RESEARCH
9 PROGRAMS. WE HAVE A PRESENTATION BY DR. SAMBRANO.

10 DR. SAMBRANO: THANK YOU, MR. CHAIRMAN.
11 GOOD MORNING, EVERYONE. SO I HAVE A SLIDE
12 PRESENTATION. WE ALSO PROVIDED A MEMO THAT PROVIDES
13 SOME BACKGROUND AND RATIONALE FOR WHAT WE ARE
14 PROPOSING AS WELL AS THE ACTION THAT WE'RE
15 REQUESTING TODAY.

16 JUST TO MAKE SURE THAT EVERYBODY IS AWARE
17 OF HOW IT IS THAT WE GOT THIS ITEM TO THIS POINT, A
18 DISCUSSION REGARDING ADDING GENE THERAPY APPROACHES
19 TO OUR CADRE OF ELIGIBLE PROJECTS BEGAN OCTOBER 11TH
20 AT A SCIENCE SUBCOMMITTEE, WHICH THEN LED TO A
21 PROPOSAL THAT WE PRESENTED AT A FULL MEETING ON
22 OCTOBER 18TH.

23 AND SO AT THAT TIME THE BOARD RECOMMENDED
24 THAT WE MORE CLEARLY DEFINE THE SCOPE OF ELIGIBLE
25 GENE THERAPY PROJECTS AND ALSO WE INCLUDE ONLY THOSE

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1 THAT HAVE A REGENERATIVE MEDICINE AIM. SO WE
2 REVISED THE PROPOSAL, BROUGHT IT TO THE SCIENCE
3 SUBCOMMITTEE ON NOVEMBER 8TH. IT WAS DISCUSSED
4 THERE AND GENERALLY APPROVED. THE SUBCOMMITTEE DID
5 RECOMMEND SOME CHANGES REGARDING THE PROCESS FOR HOW
6 THE PROJECTS WOULD BE ASSESSED FOR A VITAL RESEARCH
7 OPPORTUNITY. AND SO WE ARE NOW BRINGING THAT
8 PROPOSAL TO YOU TODAY.

9 SO JUST WITH THE SLIDE DECK, I WANT TO
10 POINT OUT JUST A COUPLE OF THINGS THAT ARE IMPORTANT
11 IN TERMS OF WHAT IT IS WE ARE PROPOSING AND WHAT WE
12 ARE NOT. SO THE PROPOSAL HERE IS FOR GENE THERAPY
13 THAT DOESN'T INCLUDE STEM CELLS. SO WE ALREADY
14 ALLOW GENE THERAPY PROJECTS THAT INVOLVE A STEM OR
15 PROGENITOR CELL SIMPLY BY THE FACT THAT THEY HAVE
16 STEM OR PROGENITOR CELLS AS PART OF THE PROJECT.

17 SO IN LOOKING TO EXPAND IT TO THOSE THAT
18 DO NOT INCLUDE STEM CELLS AND, LIKE ANY AREA OUTSIDE
19 OF THE STEM CELL AND PROGENITOR CELL PURVIEW, THIS
20 REQUIRES A VOTE BY THE GRANTS WORKING GROUP THAT
21 WOULD DEEM SUCH PROJECTS TO BE A VITAL RESEARCH
22 OPPORTUNITY. AND I'LL SPEAK A LITTLE BIT TO THE
23 PROCESS AT THE END.

24 SO THE WAY THAT WE ARE DEFINING
25 ELIGIBILITY IS SHOWN ON SLIDE 3. THERE ARE THREE

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1 BASIC CONDITIONS THAT NEED TO BE MET. SO IF
2 SOMEBODY IS PROPOSING A GENE THERAPY APPROACH, THE
3 FIRST IS THAT IT EITHER TARGETS A STEM CELL, WHICH
4 WE KNOW IS ALREADY ELIGIBLE OR ANY OTHER SOMATIC
5 CELL IF IT'S DEEMED A VITAL RESEARCH OPPORTUNITY BY
6 THE GRANTS WORKING GROUP.

7 SECONDLY, THAT THE APPROACH IS INTENDED TO
8 REPLACE, REGENERATE, OR REPAIR THE FUNCTION OF AGED,
9 DISEASED, DAMAGED, OR DEFECTIVE CELLS, TISSUES,
10 AND/OR ORGAN. THIS BASICALLY CONSTITUTES THE
11 DEFINITION OF REGENERATIVE MEDICINE AND BRINGS THAT
12 AS A REQUIREMENT.

13 AND THEN, LASTLY, THAT IT'S BEING
14 DEVELOPED FOR A RARE, UNMET MEDICAL NEED UNLIKELY TO
15 RECEIVE FUNDING FROM OTHER SOURCES. SO IT MUST MEET
16 THOSE THREE CRITERIA IN ORDER TO BE ELIGIBLE.

17 ON THE NEXT SLIDE WE HAVE ALSO PROVIDED A
18 DEFINITION OF GENE THERAPY AS FAR AS CIRM IS
19 CONCERNED AND FOR THE PURPOSE OF THESE
20 SOLICITATIONS.

21 SO AT CIRM WE ARE CONSIDERING GENE THERAPY
22 TO MEAN A HUMAN THERAPEUTIC INTERVENTION THAT'S
23 INTENDED TO DO EITHER, ONE, ALTER THE GENOMIC
24 SEQUENCE OF CELLS OR THE CELLULAR GENOME; OR, TWO,
25 ALTER THE CELLULAR LINEAGE VIA GENE DELIVERY. AND

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1 SO HERE WE ARE REFERRING TO DIRECT LINEAGE
2 REPROGRAMMING, WHICH IS A TECHNOLOGY THAT EVOLVED
3 OUT OF SOME BASIC STEM CELL BIOLOGY.

4 THE TYPES OF INTERVENTIONS THAT WE ARE
5 THINKING ABOUT CAN INCLUDE STRATEGIES TO REPAIR
6 DISEASE-CAUSING GENE SEQUENCE, REMOVE OR INACTIVATE
7 THE DISEASE-CAUSING GENE, OR INTRODUCE NEW OR
8 MODIFIED GENES THAT AUGMENT THERAPEUTIC POTENTIAL
9 FOR TARGET CELLS SUCH AS CAR-T'S AND OTHER
10 APPROACHES LIKE THAT.

11 AND SO, FINALLY, THE PROCESS FOR HOW WE
12 WOULD ESTABLISH PROJECTS THAT COME TO US AS A VITAL
13 RESEARCH OPPORTUNITY. SO APPLICATIONS THAT MEET
14 OTHERWISE THE GENERAL GENE THERAPY ELIGIBILITY
15 CRITERIA WOULD BE ACCEPTED FOR GRANTS WORKING GROUP
16 REVIEW. AND THEN FOLLOWING THE REVIEW AND AT THAT
17 MEETING, THE GRANTS WORKING GROUP WOULD TAKE A VOTE
18 ON WHETHER THEY FEEL THAT THE APPLICATION REPRESENTS
19 A VITAL RESEARCH OPPORTUNITY. AND ASSUMING THAT THE
20 VOTE IS POSITIVE, THEN IT WOULD MOVE FORWARD. IF
21 THE GWG DOES NOT AGREE THAT THE APPLICATION IS A
22 VITAL RESEARCH OPPORTUNITY, THEN THAT APPLICATION
23 WOULD BE WITHDRAWN FROM FURTHER CONSIDERATION.

24 THAT CONCLUDES MY PRESENTATION AND HAPPY
25 TO TAKE ANY QUESTIONS. MR. CHAIRMAN.

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1 CHAIRMAN THOMAS: THANK YOU, DR. SAMBRANO.
2 THESE RECOMMENDED CHANGES ARE REFLECTED IN THE
3 AMENDED CLIN AND TRAN CONCEPT PLANS ON THE AGENDA.
4 SO IN ORDER TO ENACT THESE, YOU NEED A VOTE OF THE
5 FULL BOARD TO TAKE THE RECOMMENDATIONS OF THE
6 SCIENCE SUBCOMMITTEE AND SO ENACT. DO I HEAR A
7 MOTION TO THAT EFFECT?

8 DR. MARTIN: SO MOVED. THIS IS DAVE.

9 CHAIRMAN THOMAS: THANK YOU, DAVE.

10 DR. LUBIN: I'LL SECOND IT.

11 CHAIRMAN THOMAS: MOVED BY DR. MARTIN,
12 SECONDED BY DR. LUBIN. IS THERE DISCUSSION BY
13 MEMBERS OF THE BOARD?

14 DR. MARTIN: I SECONDED IT BECAUSE I THINK
15 THIS IS A SIGNIFICANT IMPROVEMENT OVER WHAT WE SAW A
16 MONTH AGO OR LAST MONTH. I LIKE IT. I THINK IT'S
17 THOROUGH.

18 ONE OF THE THINGS THAT I WAS HAVING
19 CONCERNS ABOUT AT THE OCTOBER 18TH MEETING WAS THE
20 REQUIREMENT THAT THIS BE IN VIVO GENE THERAPY. AND
21 THAT WAS IN MY MIND JUST NOT PRACTICAL, NOT BROAD
22 ENOUGH IF WE ARE ABLE TO HAVE GENE THERAPY SUPPORT.
23 AND THIS MAKES IT VERY CLEAR THAT THAT'S NOT A
24 LIMITATION OR RESTRICTION WITHIN THE DEFINITION.

25 SO I LIKE THE DESCRIPTION. MAY WANT`TO

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1 USE IT MYSELF FOR SOME OTHER PURPOSE.

2 CHAIRMAN THOMAS: THANK YOU, DR. MARTIN.

3 ARE THERE ANY OTHER COMMENTS BY MEMBERS OF THE
4 BOARD?

5 DR. STEWARD: THIS IS OS. IF I COULD JUST
6 COMMENT, J.T.

7 CHAIRMAN THOMAS: ABSOLUTELY.

8 DR. STEWARD: I JUST WANTED TO SAY THANK
9 YOU TO CIRM STAFF AND EVERYONE WHO WORKED HARD AND
10 VERY THOUGHTFULLY ON PUTTING THIS TOGETHER. I AGREE
11 I THINK IT'S VERY CLEAR WHAT IS IN AND WHAT ISN'T IN
12 SCOPE. BUT I THINK GOING FORWARD THAT WILL HELP
13 BOTH CIRM MAKE DECISIONS ABOUT ELIGIBILITY, BUT ALSO
14 INFORM THE GRANTS WORKING GROUP DIRECTLY ABOUT WHAT
15 IT IS THAT WE ARE THINKING IN OPENING UP THIS
16 POSSIBILITY. SO THANKS TO CIRM STAFF.

17 CHAIRMAN THOMAS: THANK YOU, DR. STEWARD.
18 ANY OTHER COMMENTS BY MEMBERS OF THE BOARD? HEARING
19 NONE, ARE THERE ANY COMMENTS BY MEMBERS OF THE
20 PUBLIC AT ANY OF THE NOTICED LOCATIONS? HEARING
21 NONE, MARIA, WILL YOU PLEASE CALL THE ROLL.

22 MS. BONNEVILLE: GEORGE BLUMENTHAL. LINDA
23 BOXER.

24 DR. BOXER: YES.

25 MS. BONNEVILLE: DAVID BRENNER. LARS

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1 BERGLUND.
2 DR. BERGLUND: YES.
3 MS. BONNEVILLE: DEBORAH DEAS.
4 DR. DEAS: YES.
5 MS. BONNEVILLE: ANNE-MARIE DULIEGE.
6 ANNE-MARIE, YOU'RE ON MUTE. WE'LL COME BACK TO
7 ANNE-MARIE. JUDY GASSON.
8 DR. GASSON: YES.
9 MS. BONNEVILLE: BERT LUBIN.
10 DR. LUBIN: YES.
11 MS. BONNEVILLE: DAVID HIGGINS.
12 DR. HIGGINS: YES.
13 MS. BONNEVILLE: STEPHEN JUELSGAARD.
14 MR. JUELSGAARD: YES.
15 MS. BONNEVILLE: SHERRY LANSING.
16 LINDA MALKAS.
17 DR. MALKAS: YES.
18 MS. BONNEVILLE: DAVE MARTIN.
19 DR. MARTIN: YES.
20 MS. BONNEVILLE: LEON FINE.
21 DR. FINE: YES.
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23 PADILLA. JOE PANETTA.
24 MR. PANETTA: YES.
25 MS. BONNEVILLE: FRANCISCO PRIETO.

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1 DR. PRIETO: AYE.
2 MS. BONNEVILLE: ROBERT QUINT.
3 DR. QUINT: YES.
4 MS. BONNEVILLE: AL ROWLETT. SUZANNE
5 SANDMEYER. JEFF SHEEHY.
6 MR. SHEEHY: YES.
7 MS. BONNEVILLE: OSWALD STEWARD.
8 DR. STEWARD: YES.
9 MS. BONNEVILLE: ANNE-MARIE, IS THAT YOU?
10 DR. DULIEGE: YES. I'M HERE.
11 MS. BONNEVILLE: THANK YOU. AND IS THAT A
12 YES VOTE?
13 DR. DULIEGE: YES, IT'S A YES VOTE.
14 MS. BONNEVILLE: JONATHAN THOMAS.
15 CHAIRMAN THOMAS: YES.
16 MS. BONNEVILLE: ART TORRES.
17 MR. TORRES: AYE.
18 MS. BONNEVILLE: KRISTINA VUORI.
19 DR. VUORI: YES.
20 MS. BONNEVILLE: DIANE WINOKUR.
21 MS. WINOKUR: YES.
22 MS. BONNEVILLE: THANK YOU. THE MOTION
23 CARRIES.
24 CHAIRMAN THOMAS: THANK YOU, MARIA. AND
25 I'D LIKE TO ECHO DR. STEWARD'S COMMENTS TO DR.

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1 SAMBRANO AND THE TEAM. THANKS VERY MUCH FOR YOUR
2 HARD WORK ON THIS ITEM.

3 WE'RE GOING TO GO NOW BACK TO ITEM NO. 3,
4 CONSIDERATION OF APPLICATIONS SUBMITTED IN RESPONSE
5 TO CLINICAL TRIAL STAGE PROJECTS, CLIN1, 2, OR 3.
6 GOING INTO PROGRAMMATIC REVIEW, I'LL TURN THE
7 MEETING OVER TO MR. SHEEHY.

8 SUPERVISOR SHEEHY: THANK YOU, CHAIRMAN
9 THOMAS. I BELIEVE WE HAVE ONE APPLICATION TO
10 CONSIDER TODAY. DR. SAMBRANO, ARE YOU GOING TO TAKE
11 US THROUGH THIS?

12 DR. SAMBRANO: IT'S GOING TO BE DR. PATEL.

13 CHAIRMAN SHEEHY: OH, GREAT. DR. PATEL,
14 PLEASE.

15 DR. PATEL: THANKS, JEFF.

16 FIRST OF ALL, I'D LIKE TO -- I HOPE
17 EVERYBODY AND THEIR FAMILIES ARE SAFE FROM THE FIRES
18 IN THE STATE. AND IT'S A PLEASURE TO INTRODUCE THE
19 CLINICAL APPLICATION FOR YOUR REVIEW TODAY.

20 SO, AS YOU KNOW, THE CLINICAL PROGRAM
21 CONSISTS OF THREE FUNDING OPPORTUNITIES. TODAY
22 THERE'S ONE APPLICATION UP FOR REVIEW, AND THAT'S A
23 CLIN1 APPLICATION. THIS IS AN IND-ENABLING PROJECT.

24 SO THE GWG, WHEN THEY SCORE THESE CLIN
25 APPLICATIONS, THEY USE A THREE-TIER SCORING SYSTEM.

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1 A SCORE OF 1 WOULD INDICATE THAT THE APPLICATION HAS
2 EXCEPTIONAL MERIT AND WARRANTS FUNDING. A SCORE OF
3 2 WOULD INDICATE THAT IT NEEDS IMPROVEMENT AND DOES
4 NOT WARRANT FUNDING AT THIS TIME, BUT CAN BE
5 RESUBMITTED TO ADDRESS THE AREAS OF IMPROVEMENT.
6 AND, FINALLY, IF THE APPLICATION IS SUFFICIENTLY
7 FLAWED THAT IT DOES NOT WARRANT FUNDING, IT GETS A
8 SCORE OF 3, AND THAT APPLICATION CANNOT BE
9 RESUBMITTED FOR AT LEAST SIX MONTHS.

10 TO GIVE YOU AN IDEA AS TO WHERE WE'RE AT
11 IN TERMS OF OUR ANNUAL BUDGET, AS YOU KNOW, YOU
12 ALLOCATED \$130 MILLION TO THE CLIN PROGRAM FOR THIS
13 YEAR EXCEPT THE TYPO UP TOP SAYS END OF SEPTEMBER.
14 IT ACTUALLY MEANS END OF OCTOBER. WE APOLOGIZE FOR
15 THAT.

16 TO DATE YOU APPROVED AWARDS TOTALING
17 ROUGHLY \$90 MILLION. THE AWARD THAT IS UP FOR
18 CONSIDERATION TODAY IS \$5.7 MILLION BUDGET. IF YOU
19 WERE TO APPROVE THAT ONE, THERE WOULD BE 34.6
20 MILLION LEFT AS AN UNOBLIGATED BALANCE FOR THE CLIN
21 PROGRAM FOR THIS YEAR. AND THERE WILL BE ONE MORE
22 BOARD MEETING IN DECEMBER, AS YOU KNOW.

23 CIRM SETS INTERNAL TARGETS ON AN ANNUAL
24 BASIS TO HELP US MEET THE BIG SIX GOALS. WE HAD AN
25 ANNUAL TARGET OF 12 CLIN2S. WE HAVE FUNDED SIX TO

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1 DATE. WE HAD AN ANNUAL TARGET OF FOUR CLINIS. WE
2 HAVE FUNDED FIVE TO DATE; AND IF YOU WERE TO FUND
3 TODAY'S, THAT WOULD MAKE THAT SIX.

4 SO TODAY'S APPLICATION IS CLIN1-11404.
5 IT'S A LATE STAGE PRECLINICAL THERAPY FOR
6 MYELOMENINGOCELE ALSO KNOWN AS SPINA BIFIDA.
7 THERAPY ITSELF IS MESENCHYMAL STEM CELLS SEATED ONTO
8 A COLLAGEN MATRIX. SO THIS WOULD BE A CELL/DEVICE
9 COMBINATION PRODUCT. THE INDICATION IS PRENATALLY
10 DIAGNOSED MYELOMENINGOCELE WHICH IS THE MOST SEVERE
11 FORM OF SPINA BIFIDA. AND THE OVERALL GOAL FOR THIS
12 PROJECT IS TO SUBMIT AN IND TO ENABLE CLINICAL
13 TESTING. AND ALONG THE WAY, THE ACTIVITIES THEY'RE
14 PROPOSING ARE PRODUCT MANUFACTURING OPTIMIZATION,
15 DOING SAFETY AND EFFICACY STUDIES AS REQUIRED BY THE
16 FDA, AND THAN SUBMITTING THE IND.

17 THEY'RE REQUESTING \$5.7 MILLION IN FUNDING
18 FOR THIS PROJECT, AND THERE'S ZERO DOLLARS
19 CO-FUNDING. JUST TO NOTE, THAT THE MAXIMUM FUNDS
20 ALLOWABLE FOR THIS CATEGORY IS \$6 MILLION.

21 SOME PROGRAMMATIC INFORMATION THAT MAY BE
22 HELPFUL FOR YOUR REVIEW. ABOUT 1500 BABIES ARE BORN
23 WITH SPINA BIFIDA IN THE U.S. EVERY YEAR. AND THIS
24 DISEASE DISPROPORTIONATELY AFFECTS CHILDREN OF
25 HISPANIC OR LATINO DESCENT, WHICH IS IMPORTANT FOR

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1 THE STATE OF CALIFORNIA. MYELOMENINGOCELE IS THE
2 MOST SEVERE FORM OF SPINA BIFIDA, AND IT RESULTS IN
3 PARALYSIS BELOW THE SPINAL OPENING, AND IT ALSO
4 RESULTS IN OTHER CNS COMPLICATIONS SUCH AS
5 MALFORMATIONS AND HYDROCEPHALUS. SO BASICALLY THE
6 BOTTOM LINE IS THAT THESE PATIENTS WILL REQUIRE
7 EXTENSIVE ASSISTANCE WITH DISABILITIES AS WELL AS
8 CONTINUAL MEDICAL CARE FOR THEIR LIVES.

9 THE VALUE PROPOSITION OF THIS APPROACH IS
10 THAT UNTIL RECENTLY THE MOST COMMON TREATMENT FOR
11 SPINA BIFIDA WAS POSTNATAL SURGICAL CLOSURE. AND
12 THE MAIN INTENT WAS TO PREVENT INFECTION, AND IT WAS
13 NOT DESIGNED TO RESTORE ANY MOTOR FUNCTION.

14 IN THE PAST DECADE, THERE WAS A STUDY
15 CONDUCTED BY THE NIH APTLY NAMED THE MOM STUDY,
16 WHICH WAS THE MANAGEMENT OF MYELOMENINGOCELE WHERE
17 THEY STUDIED WHETHER THEY CAN DO FETAL SURGICAL
18 CLOSURE OF SPINA BIFIDA AND WHETHER THAT WOULD
19 RESULT IN ANY IMPROVEMENTS. WHAT THEY FOUND WAS IT
20 REDUCED THE NEED FOR SHUNTING, AND IT IMPROVED MOTOR
21 OUTCOME IN A SUBSET OF PATIENTS. NOW, THESE BABIES
22 STILL SUFFERED FROM DISABILITIES, BUT THERE WAS
23 IMPROVEMENT IN MOTOR FUNCTION.

24 SO THE IDEA WITH THE CURRENT APPROACH IS,
25 ONE, FETAL CLOSURE IS DONE, THEY WOULD APPLY THIS

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1 PATCH OF STEM CELLS SEATED ON A MATRIX WHICH COULD
2 ACT AS A NEUROPROTECTIVE STEM CELL THERAPY THAT CAN
3 AUGMENT THE OVERALL FETAL SURGERY AND POTENTIALLY
4 IMPROVE MOTOR OUTCOME AND HOPEFULLY REDUCE
5 DISABILITY IN THE BABIES.

6 WHY IS THIS A STEM CELL PROJECT? BECAUSE
7 IT INVOLVES PLACENTA-DERIVED MESENCHYMAL STEM CELLS.
8 CIRM IS CURRENTLY NOT FUNDING ANY CLINICAL STAGE
9 SPINA BIFIDA PROJECTS. SO THIS WILL BE THE FIRST IN
10 THIS PARTICULAR DISEASE INDICATION.

11 THIS PROJECT HAS RECEIVED PREVIOUS CIRM
12 FUNDING ON A TRANSLATIONAL AWARD, AND THE OUTCOME OF
13 THAT WAS THE SUCCESSFUL CONDUCT OF A PRE-IND
14 MEETING, WHICH THEY DID ACHIEVE, AND THAT PROJECT
15 ENDED IN AUGUST OF THIS YEAR.

16 THE GWG REVIEWED THIS APPLICATION AND
17 UNANIMOUSLY GAVE IT A SCORE OF 1 AND RECOMMENDED IT
18 FOR FUNDING BY THE BOARD. AND THE AWARD AMOUNT --
19 THE CIRM TEAM RECOMMENDATION IS TO FUND THIS AWARD
20 AND TO CONCUR WITH THE GWG RECOMMENDATION FOR THE
21 FULL AWARD AMOUNT OF \$5.67 MILLION. JEFF.

22 CHAIRMAN SHEEHY: OKAY. ARE THERE ANY
23 QUESTIONS FROM MEMBERS OF THE BOARD?

24 DR. LUBIN: THE ONLY COMMENT I WOULD
25 ADD -- THAT WAS A REALLY NICE PRESENTATION -- IS

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1 THAT THE COST OF TAKING CARE OF THESE CHILDREN, NOT
2 ONLY EMOTIONALLY FOR THE FAMILY, BUT THE ACTUAL COST
3 TO THE STATE IS (CUT OFF). SO THIS HAS AN ECONOMIC
4 BENEFIT TO THE STATE AS WELL AS TO THE FAMILIES IF
5 SUCCESSFUL.

6 CHAIRMAN SHEEHY: THANK YOU, DR. LUBIN.

7 DO WE HAVE A MOTION TO ACCEPT THE CIRM
8 TEAM RECOMMENDATION AND FUND THIS PROJECT?

9 DR. HIGGINS: I SO MOVE.

10 DR. LUBIN: I'LL SECOND IT.

11 MR. TORRES: ART.

12 CHAIRMAN SHEEHY: SECONDED BY SENATOR
13 TORRES. ARE THERE ANY OTHER BOARD COMMENTS OR
14 QUESTIONS?

15 MS. BONNEVILLE: BERT CAN'T SECOND THE
16 MOTION, SO CAN WE GET ANOTHER SECOND?

17 MR. TORRES: YEAH.

18 MS. BONNEVILLE: THANK YOU.

19 MR. TORRES: MR. CHAIRMAN, I HAD A
20 QUESTION. I'M SORRY, BERT, BUT YOU WERE CUT OFF
21 WHEN YOU SAID THE COST TO THE STATE OF TREATING A
22 CHILD PER YEAR IS WHAT?

23 DR. LUBIN: I DON'T HAVE THE NUMBER, BUT
24 IT'S EXCESSIVE. I MEAN IT'S A CHRONIC CARE,
25 COMPLICATED SITUATION FOR THE FAMILY AS WELL AS ALL

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1 THE AGENCIES THAT FUND SERVICES FOR THESE CHILDREN.

2 IT'S A VERY COMPLEX ILLNESS.

3 MR. TORRES: THANK YOU.

4 CHAIRMAN SHEEHY: ANY ADDITIONAL BOARD
5 COMMENTS OR QUESTIONS? ANY PUBLIC COMMENT? COULD
6 WE CALL THE ROLL THEN PLEASE.

7 MS. BONNEVILLE: ANNE-MARIE DULIEGE.

8 DR. DULIEGE: YES.

9 MS. BONNEVILLE: DAVID HIGGINS.

10 DR. HIGGINS: YES.

11 MS. BONNEVILLE: STEVE JUELSGAARD.

12 MR. JUELSGAARD: YES.

13 MS. BONNEVILLE: DAVE MARTIN.

14 DR. MARTIN: YES.

15 MS. BONNEVILLE: LAUREN MILLER. ADRIANA
16 PADILLA. JOE PANETTA.

17 MR. PANETTA: YES.

18 MS. BONNEVILLE: FRANCISCO PRIETO.

19 DR. PRIETO: AYE.

20 MS. BONNEVILLE: ROBERT QUINT.

21 DR. QUINT: YES.

22 MS. BONNEVILLE: AL ROWLETT. JEFF SHEEHY.

23 CHAIRMAN SHEEHY: YES.

24 MS. BONNEVILLE: OS STEWARD.

25 DR. STEWARD: YES.

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1 MS. BONNEVILLE: JONATHAN THOMAS.

2 CHAIRMAN THOMAS: YES.

3 MS. BONNEVILLE: ART TORRES.

4 MR. TORRES: AYE.

5 MS. BONNEVILLE: DIANE WINOKUR.

6 MS. WINOKUR: YES.

7 MS. BONNEVILLE: THANK YOU. THE MOTION

8 CARRIES.

9 CHAIRMAN SHEEHY: THANK YOU. THAT
10 CONCLUDES THE BUSINESS OF THE APPLICATION REVIEW
11 SUBCOMMITTEE, AND I TURN IT BACK OVER TO YOU,
12 CHAIRMAN THOMAS.

13 CHAIRMAN THOMAS: THANK YOU, MR. SHEEHY.
14 IS THERE ANY PUBLIC COMMENT ON ANY ITEMS FROM ANY OF
15 THE NOTICED LOCATIONS?

16 HEARING NONE, I'D JUST LIKE TO NOTE THIS
17 HAS BEEN A VERY DIFFICULT COUPLE OF WEEKS FOR THE
18 STATE OF CALIFORNIA AND WOULD LIKE TO JUST COMMENT
19 ON HOW MUCH WE VALUE OUR BRAVE FIRST RESPONDERS IN
20 THE POLICE DEPARTMENT AND FIRE DEPARTMENTS AND WOULD
21 LIKE TO ADJOURN THIS MEETING IN THE MEMORY OF THOSE
22 WHO LOST THEIR LIVES AT THE BORDERLINE BAR & GRILL
23 AND CAMP AND WOOLSEY FIRES.

24 SO WITHOUT FURTHER ADO, THAT CONCLUDES THE
25 BUSINESS OF THE ICOC AND APPLICATION REVIEW

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1 SUBCOMMITTEE. THIS MEETING STANDS ADJOURNED.

2 MS. BONNEVILLE: THANK YOU, EVERYONE. SEE
3 YOU IN DECEMBER.

4 (THE MEETING WAS THEN CONCLUDED AT
5 11:26 A.M.)

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REPORTER'S CERTIFICATE

I, BETH C. DRAIN, A CERTIFIED SHORTHAND REPORTER IN AND FOR THE STATE OF CALIFORNIA, HEREBY CERTIFY THAT THE FOREGOING TRANSCRIPT OF THE TELEPHONIC PROCEEDINGS BEFORE THE INDEPENDENT CITIZEN'S OVERSIGHT COMMITTEE AND THE APPLICATION REVIEW SUBCOMMITTEE OF THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE IN THE MATTER OF ITS REGULAR MEETING HELD ON NOVEMBER 15, 2018, WAS HELD AS HEREIN APPEARS AND THAT THIS IS THE ORIGINAL TRANSCRIPT THEREOF AND THAT THE STATEMENTS THAT APPEAR IN THIS TRANSCRIPT WERE REPORTED STENOGRAPHICALLY BY ME AND TRANSCRIBED BY ME. I ALSO CERTIFY THAT THIS TRANSCRIPT IS A TRUE AND ACCURATE RECORD OF THE PROCEEDING.

BETH C. DRAIN, CSR 7152
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