

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: CALIFORNIA INSTITUTE FOR  
REGENERATIVE MEDICINE  
1999 HARRISON STREET, SUITE 1650  
OAKLAND, CALIFORNIA

DATE: MARCH 23, 2017  
11 A.M.

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

FILE NO.: 2017-10

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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 CLIN1: PARTNERING OPPORTUNITY FOR LATE STAGE PRECLINICAL PROJECTS	5
4. CONSIDERATION OF APPLICATIONS SUBMITTED IN RESPONSE TO THE PARTNERING OPPORTUNITY FOR TRANSLATIONAL RESEARCH PROJECTS	11
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 CLIN1: PARTNERING OPPORTUNITY FOR LATE STAGE PRECLINICAL PROJECTS AND THE PARTNERING OPPORTUNITY FOR TRANSLATIONAL RESEARCH PROJECTS	
6. PUBLIC COMMENT	NONE
7. ADJOURNMENT	26

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THURSDAY, MARCH 23, 2017

11 A.M.

CHAIRMAN THOMAS: I'D LIKE TO WELCOME EVERYBODY -- THIS IS J.T. -- TO THE REGULAR MEETING OF THE ICOC AND THE APPLICATION REVIEW SUBCOMMITTEE FOR MARCH 2017. WE ARE BROADCASTING TO YOU FROM THE CITY OF HOPE WHERE WE'RE IN THE PROCESS TODAY OF A MOST INFORMATIVE AND INTERESTING ALPHA CLINIC SYMPOSIUM THAT THE CITY OF HOPE IS HOSTING FOR OUR ALPHA CLINIC NETWORK.

AND WE HAVE FOLKS HERE FROM MEMBERS OF THE PUBLIC. WOULD LIKE TO HAVE MARIA CALL THE ROLL.

MS. BONNEVILLE: GEORGE BLUMENTHAL. DAVID BRENNER. KEN BURTIS. DEBORAH DEAS. ANNE-MARIE DULIEGE. HOWARD FEDEROFF. JUDY GASSON. SAM HAWGOOD. DAVID HIGGINS.

DR. HIGGINS: HERE.

MS. BONNEVILLE: STEVE JUELSGAARD.

DR. JUELSGAARD: HERE.

MS. BONNEVILLE: SHERRY LANSING. KATHY LAPORTE.

MS. LAPORTE: HERE.

MS. BONNEVILLE: BERT LUBIN. SHLOMO MELMED. LAUREN MILLER.

1 MS. MILLER: HERE.  
2 MS. BONNEVILLE: LLOYD MINOR. ADRIANA  
3 PADILLA.  
4 DR. PADILLA: HERE.  
5 MS. BONNEVILLE: JOE PANETTA.  
6 MR. PANETTA: HERE.  
7 MS. BONNEVILLE: FRANCISCO PRIETO. CARMEN  
8 PULIAFITO. ROBERT QUINT.  
9 DR. QUINT: HERE.  
10 MS. BONNEVILLE: AL ROWLETT.  
11 MR. ROWLETT: HERE.  
12 MS. BONNEVILLE: JEFF SHEEHY.  
13 SUPERVISOR SHEEHY: HERE.  
14 MS. BONNEVILLE: OS STEWARD.  
15 DR. STEWARD: HERE.  
16 MS. BONNEVILLE: JONATHAN THOMAS.  
17 CHAIRMAN THOMAS: HERE.  
18 MS. BONNEVILLE: ART TORRES.  
19 MR. TORRES: HERE.  
20 MS. BONNEVILLE: KRISTINA VUORI. DIANE  
21 WINOKUR.  
22 CHAIRMAN THOMAS: THANK YOU, MARIA.  
23 MS. WINOKUR: THIS IS DIANE WINOKUR.  
24 MS. BONNEVILLE: THANK YOU, DIANE.  
25 CHAIRMAN THOMAS: WE'RE GOING TO MOVE TO

1 ITEM 3 IN THE AGENDA, WHICH IS CONSIDERATION OF  
2 APPLICATIONS SUBMITTED IN RESPONSE TO CLIN1:  
3 PARTNERING OPPORTUNITY FOR LATE STAGE PRECLINICAL  
4 PROJECTS. AT THIS POINT I WILL TURN THE MEETING  
5 OVER TO SUPERVISOR SHEEHY.

6 SUPERVISOR SHEEHY: THANK YOU, CHAIRMAN  
7 THOMAS. DR. SAMBRANO, DO YOU HAVE A PRESENTATION?

8 DR. SAMBRANO: YES, I DO.

9 SUPERVISOR SHEEHY: GREAT. THANK YOU.

10 DR. SAMBRANO: GOOD MORNING, EVERYONE.  
11 THIS IS AN APPLICATION THAT WE ARE BRINGING FOR YOUR  
12 CONSIDERATION THAT WAS REVIEWED IN JANUARY. IT WAS  
13 RESPONDING TO THE CLIN1, CLINICAL PROGRAM FOR  
14 IND-ENABLING WORK. THE APPLICANT APPEALED THAT, IT  
15 HAS NOW BEEN RESOLVED, AND SO WE'RE BRINGING THIS  
16 FOR YOUR CONSIDERATION TODAY.

17 A REMINDER OF A COUPLE OF NOTES IN THE  
18 PROCESS. WHEN WE CONDUCT THE GWG REVIEW, WE HAVE  
19 ALL MEMBERS OF THE GWG VOTE ON WHETHER THEY FELT  
20 THAT THE REVIEW WAS CONDUCTED IN A SCIENTIFIC  
21 RIGOROUS WAY, THAT THERE WAS SUFFICIENT TIME ALLOWED  
22 FOR ALL VIEWPOINTS TO BE HEARD, AND THAT THE SCORES  
23 REFLECT THE RECOMMENDATION OF THE GWG. OUR PATIENT  
24 ADVOCATE MEMBERS WHO ARE ALSO MEMBERS OF THIS BOARD  
25 ALSO VOTE ON WHETHER THEY FEEL THE REVIEW IS CARRIED

1 OUT IN A FAIR MANNER AND WAS FREE FROM UNDUE BIAS.  
2 IN THIS CASE, AS WAS FOR ALL THE APPLICATIONS  
3 REVIEWED DURING THIS CYCLE, THE VOTE WAS UNANIMOUS  
4 IN FAVOR OF BOTH STATEMENTS THAT WERE CONSIDERED ON  
5 THAT DAY.

6 A REMINDER OF THE SCORING SYSTEM FOR THESE  
7 APPLICATIONS. THESE ARE SCORED ON A 1-2-3 BASIS. A  
8 SCORE OF 1 MEANS THAT THEY HAVE EXCEPTIONAL MERIT  
9 AND WARRANT FUNDING; SCORE OF 2 MEANS THE  
10 APPLICATION NEEDS IMPROVEMENT, BUT CAN BE SUBMITTED  
11 TO ADDRESS THE AREAS FOR IMPROVEMENT; AND A SCORE OF  
12 3 MEANS THAT THE APPLICATION IS SUFFICIENTLY FLAWED  
13 THAT IT DOESN'T WARRANT FUNDING AND THIS PROJECT  
14 SHOULD NOT BE SUBMITTED FOR SIX MONTHS.

15 THE APPLICATION THAT IS BEING CONSIDERED  
16 IS CLIN1-09759. THIS IS A PRECLINICAL DEVELOPMENT  
17 PROJECT FOR A CELL THERAPY TO TREAT PARKINSON'S  
18 DISEASE. THE THERAPY IS A HUMAN EMBRYONIC STEM  
19 CELL-DERIVED NEUROPROGENITOR CELL THAT EXPRESSES  
20 MEF2C, A TRANSCRIPTION FACTOR. IT IS FOR PATIENTS  
21 WITH MODERATE TO SEVERE PARKINSON'S DISEASE.

22 THE GOAL OF THE PROJECT IS TO COMPLETE  
23 PRECLINICAL ACTIVITIES THAT WILL ENABLE THEM TO FILE  
24 AN IND FOR TESTING THE CELL THERAPY PRODUCT IN THE  
25 CLINIC. SOME OF THE MAJOR PROPOSED ACTIVITIES

1 INCLUDE MANUFACTURING THE PRODUCT, SOME PRECLINICAL  
2 DOSE RESPONSE EFFICACY, TOXICITY AND SAFETY STUDIES,  
3 AND, OF COURSE, TO PREPARE AND FILE THE IND.

4 THE REQUESTED AMOUNT IS 4.8 MILLION FOR  
5 THIS APPLICATION.

6 THE APPLICATION UNDERWENT A BUDGET REVIEW  
7 FOR THE ACTIVITIES THAT WERE PROPOSED AND THE COSTS  
8 THAT WERE ASSOCIATED WITH THAT. IT PASSED THAT.  
9 HOWEVER, AT THE GWG REVIEW, REVIEWERS SCORED THIS A  
10 3, WHICH MEANS THEY DO NOT FEEL IT WARRANTS FUNDING,  
11 AND THE VOTE ON THIS WAS UNANIMOUS, THIRTEEN MEMBERS  
12 OF THE GWG ALL SCORING A SCORE OF 3. THE CIRM TEAM  
13 ALSO, IN REVIEWING AND ASSESSING THE PROCESS OF THE  
14 REVIEW, AGREES WITH THE DO NOT FUND RECOMMENDATION  
15 IN THIS CASE. MR. SHEEHY.

16 SUPERVISOR SHEEHY: THANK YOU, DR.  
17 SAMBRANO. DO I HAVE A MOTION TO EITHER FUND OR NOT  
18 FUND THIS APPLICATION?

19 CHAIRMAN THOMAS: I MOVE THAT WE NOT FUND.

20 SUPERVISOR SHEEHY: DO I HAVE A SECOND?

21 MS. LAPORTE: I SECOND THAT.

22 SUPERVISOR SHEEHY: DO WE HAVE ANY  
23 DISCUSSION? DO WE HAVE ANY PUBLIC COMMENT? I THINK  
24 THERE'S INDIVIDUALS IN LOS ANGELES, MEMBERS OF THE  
25 PUBLIC.

1 CHAIRMAN THOMAS: NO PUBLIC COMMENT HERE,  
2 JEFF.

3 SUPERVISOR SHEEHY: GREAT. THANK YOU.  
4 THEN, MARIA, CAN WE HAVE A VOTE?

5 MS. BONNEVILLE: YES.

6 ANNE-MARIE DULIEGE. DAVID HIGGINS.

7 DR. HIGGINS: CAN I SUBMIT MY VOTE WITH  
8 COMMENTARY, OR DO I HAVE TO DO THAT OUTSIDE? VERY  
9 BRIEF COMMENT, IS THAT POSSIBLE?

10 MR. TORRES: I SUGGEST WE SUSPEND THE ROLL  
11 CALL AND LET DAVID SPEAK, THEN PROCEED.

12 DR. HIGGINS: I'M SORRY FOR THAT. SO THIS  
13 IS DAVID HIGGINS AT CITY OF HOPE. I THINK IT  
14 PROBABLY GOES WITHOUT SAYING THAT I HAVE A VESTED  
15 INTEREST IN THIS AREA OF RESEARCH AND THIS AREA OF  
16 FUNDING FOR CIRM. AND I ALSO WANT TO POINT OUT THAT  
17 THE APPLICANT IS A WORLD-CLASS SCIENTIST THAT HAS A  
18 LONG CAREER OF TOPNOTCH SCIENCE AND CARE FOR  
19 PATIENTS. AND THAT IS NOT IN QUESTION.

20 I THINK IT ALSO GOES WITHOUT SAYING THAT  
21 CIRM'S BEST INTEREST IS TO FUND THE LARGEST NUMBER  
22 OF THE BEST PROPOSALS THAT WE CAN FIND IN ORDER TO  
23 GET NEW THERAPIES AND EVEN CURES FOR DISEASES,  
24 INCLUDING PARKINSON'S. SO IT'S IN OUR BEST INTEREST  
25 TO FIND AND FUND THE BEST OF THE BEST. AND IT'S MY



1 HOPE IN THIS PARTICULAR CASE THAT THE APPLICANTS  
2 WILL CIRCLE BACK AROUND WITH CIRM, LOOK AT THE GWG'S  
3 COMMENTS, AND REVIEW WHAT NEEDS TO BE DONE, AND I  
4 HOPE TO SEE A RESUBMISSION FROM THEM IN THE FUTURE.  
5 THAT'S ALL I'M GOING TO SAY.

6 SUPERVISOR SHEEHY: THANK YOU. DO WE HAVE  
7 ANY OTHER BOARD COMMENT? AGAIN, DO WE HAVE ANY  
8 PUBLIC COMMENT? MS. BONNEVILLE, COULD YOU CALL THE  
9 ROLL PLEASE.

10 MS. BONNEVILLE: ANNE-MARIE DULIEGE.  
11 DAVID HIGGINS.

12 DR. HIGGINS: NO.

13 DR. JUELSGAARD: COULD SOMEBODY REPEAT THE  
14 MOTION JUST SO WE'RE CLEAR WHAT WE'RE VOTING ON?

15 SUPERVISOR SHEEHY: SURE. LET'S START  
16 OVER FROM THE TOP. THE MOTION IS TO NOT FUND  
17 APPLICATION 09759. THE MOTION WAS MADE BY CHAIRMAN  
18 THOMAS AND SECONDED BY KATHY LAPORTE.

19 SO WE'VE TAKEN BOARD COMMENT AND PUBLIC  
20 COMMENT. SO AT THIS POINT WE SHALL CALL THE ROLL.

21 MS. BONNEVILLE: ANNE-MARIE DULIEGE.  
22 DAVID HIGGINS.

23 DR. HIGGINS: PARDON MY CONFUSION. I VOTE  
24 YES.

25 MS. BONNEVILLE: STEVE JUELSGAARD.

1 DR. JUELSGAARD: YES.  
2 MS. BONNEVILLE: KATHY LAPORTE.  
3 MS. LAPORTE: YES.  
4 MS. BONNEVILLE: LAUREN MILLER.  
5 MS. MILLER: YES.  
6 MS. BONNEVILLE: ADRIANA PADILLA.  
7 DR. PADILLA: YES.  
8 MS. BONNEVILLE: JOE PANETTA.  
9 MR. PANETTA: YES.  
10 MS. BONNEVILLE: FRANCISCO PRIETO.  
11 DR. PRIETO: AYE.  
12 MS. BONNEVILLE: ROBERT QUINT.  
13 DR. QUINT: YES.  
14 MS. BONNEVILLE: AL ROWLETT.  
15 MR. ROWLETT: YES.  
16 MS. BONNEVILLE: JEFF SHEEHY.  
17 SUPERVISOR SHEEHY: YES.  
18 MS. BONNEVILLE: JONATHAN THOMAS.  
19 CHAIRMAN THOMAS: YES.  
20 MS. BONNEVILLE: ART TORRES.  
21 MR. TORRES: AYE.  
22 MS. BONNEVILLE: DIANE WINOKUR.  
23 MS. WINOKUR: YES.  
24 MS. BONNEVILLE: THANK YOU. THE MOTION  
25 CARRIES.

1 SUPERVISOR SHEEHY: THANK YOU.

2 DR. STEWARD: AM I IN CONFLICT ON THIS  
3 ONE?

4 MS. BONNEVILLE: YES.

5 DR. STEWARD: OKAY. THANK YOU.

6 SUPERVISOR SHEEHY: SO ITEM 4,  
7 CONSIDERATION OF APPLICATIONS SUBMITTED IN RESPONSE  
8 TO THE PARTNERING OPPORTUNITY FOR TRANSLATIONAL  
9 RESEARCH PROJECTS.

10 DR. SAMBRANO: THANK YOU, MR. SHEEHY.

11 SO WE'RE BRINGING FOR YOUR CONSIDERATION  
12 APPLICATIONS RESPONDING TO THE TRANSLATIONAL  
13 RESEARCH PROGRAM. THIS IS AN OPPORTUNITY THAT IS  
14 AVAILABLE NOW THREE TIMES A YEAR OR EVERY FOUR  
15 MONTHS. AND THE DEADLINE -- BECAUSE IT'S ABOUT  
16 EVERY FOUR MONTHS, THE DEADLINES COME TOO LATE AFTER  
17 THE BOARD MEETING THAT CONSIDERS THESE APPLICATIONS.  
18 SO THE NEXT DEADLINE IS COMING UP APRIL 5TH. THAT'S  
19 JUST A NOTE TO BE AWARE OF.

20 THE OBJECTIVE OF THIS PROGRAM, AS YOU  
21 KNOW, IS TO SUPPORT PROMISING STEM CELL-BASED  
22 PROJECTS THAT WILL ACCELERATE COMPLETION OF THOSE  
23 TRANSLATIONAL STAGE ACTIVITIES THAT WILL ALLOW  
24 PROJECTS TO ADVANCE TO CLINICAL STUDY OR IN THE CASE  
25 OF TOOLS TO BE COMMERCIALIZED FOR BROAD END USE.

1           THERE ARE A VARIETY OF PRODUCTS THAT CAN  
2           COME IN UNDER A TRAN AWARD: A THERAPEUTIC, A  
3           DIAGNOSTIC, A MEDICAL DEVICE OR A TOOL. THE PROGRAM  
4           ALLOWS FOR DIFFERENT -- OR BECAUSE THE TRAN  
5           ACTIVITIES DIFFER FOR EACH OF THESE PRODUCT TYPES,  
6           THERE ARE DIFFERENT FUNDING CAPS FOR THESE PROGRAMS  
7           AS WELL AS A TIME THAT'S ALLOWED TO COMPLETE THOSE  
8           ACTIVITIES. FOR A TOOL A CAP OF ONE MILLION UP TO A  
9           THERAPEUTIC WHICH ALLOWS TWO TO FOUR MILLION,  
10          DEPENDING ON WHETHER IT'S A CELL THERAPY OR SMALL  
11          MOLECULE. THERE ARE DIFFERENCES THERE AND SOME  
12          DIFFERENCES IN THE APPLICATION.

13                   HOWEVER, IN GENERAL, WHAT WE'RE LOOKING  
14          FOR ARE PRODUCTS THAT ARE AT THE STAGE WHERE THEY'VE  
15          DEMONSTRATED A PROOF OF CONCEPT. FOR A THERAPEUTIC  
16          IT MEANS THAT THEY'VE IDENTIFIED A SINGLE  
17          THERAPEUTIC CANDIDATE WITH DISEASE MODIFYING  
18          ACTIVITY; OR FOR SOMETHING LIKE A TOOL OR A DEVICE,  
19          THAT THEY HAVE A PROTOTYPE DEVELOPED IN ORDER TO  
20          DEMONSTRATE THE PROOF OF CONCEPT. SO THAT'S THE  
21          STAGE OF READINESS THAT WE ARE LOOKING FOR FOR  
22          PROJECTS THAT ARE COMING IN.

23                   THE TRANSLATION ACTIVITIES THAT THEY  
24          CONDUCT OVER THE APPROXIMATE TWO YEARS OF THE AWARD  
25          SHOULD LEAD THEM TO COMPLETING A PRE-IND MEETING OR

1 A PRESUBMISSION MEETING WITH THE FDA, DEPENDING ON  
2 THE PRODUCT TYPE; OR IF IT'S A TOOL, TO TRANSFER IT  
3 TO MANUFACTURING FOR COMMERCIALIZATION OF THAT  
4 PRODUCT.

5 THE REVIEW CRITERIA THAT WE APPLIED TO THE  
6 TRANSLATION PROGRAM ARE THE SAME AS FOR MOST OF OUR  
7 COMPETITIONS. DOES THE PROJECT HOLD THE NECESSARY  
8 SIGNIFICANCE AND POTENTIAL FOR IMPACT? THAT IS,  
9 WHAT VALUE DOES THIS PRODUCT POTENTIALLY BRING WITH  
10 THE VALUE PROPOSITION OVERALL TO PATIENTS OR TO THE  
11 RESEARCH COMMUNITY? IS THE RATIONALE SOUND? THAT  
12 IS, IS THIS SOMETHING THAT MAKES SENSE? IS THE  
13 PROJECT WELL-PLANNED AND DESIGNED? AND IS THE  
14 PROJECT FEASIBLE, INCLUDING DO THEY HAVE AN  
15 APPROPRIATE TEAM TO CONDUCT THE WORK? DO THEY HAVE  
16 THE RESOURCES AVAILABLE AND THE ENVIRONMENT TO  
17 CONDUCT THE WORK?

18 THE SCORING SYSTEM THAT WE UTILIZE IN THE  
19 TRAN IS DIFFERENT FROM THE CLINICAL PROGRAM. HERE  
20 WE USE A RANGE OF 1 TO 100, WITH A SCORE OF 85 TO  
21 100 BEING RECOMMENDED FOR A FUNDING TIER, AND  
22 ANYTHING BELOW 85 IS NOT RECOMMENDED FOR FUNDING.  
23 AND WE UTILIZE THE MEDIAN IN ORDER TO ASSIGN THE  
24 FINAL INDIVIDUAL SCORE, THE MEDIAN FROM ALL THE  
25 INDIVIDUAL GWG MEMBERS THAT SCORE.

1 THE RECOMMENDATIONS THAT CAME OUT OF THIS  
2 PARTICULAR CYCLE FOR TRAN APPLICATIONS ARE AS  
3 FOLLOWS. THERE ARE TWO APPLICATIONS THAT WERE  
4 RECOMMENDED THAT ARE IN THE TOP TIER THAT TOTAL TO  
5 AN AMOUNT OF ABOUT FOUR MILLION. THERE ARE, BECAUSE  
6 THIS IS THE FIRST CYCLE THIS YEAR, PLENTY OF FUNDS  
7 AVAILABLE TO COVER THE AMOUNT OF THOSE TWO  
8 APPLICATIONS. OF COURSE, JUST A NOTE THAT THE FINAL  
9 AWARD AMOUNT WOULD NOT EXCEED THE AMOUNT SHOWN  
10 BECAUSE WE DO GO THROUGH AN ASSESSMENT OF ALLOWABLE  
11 COSTS AND ACTIVITIES, AND IN SOME CASES THAT AMOUNT  
12 MAY BE REDUCED.

13 AS PREVIOUSLY NOTED, THE GWG VOTE ON THE  
14 REVIEW PROCESS. WE CONDUCT THAT HERE TOO. AND,  
15 AGAIN, ON THIS REVIEW, IT WAS A UNANIMOUS VOTE.

16 AND THEN, FINALLY, I'M GOING TO GIVE YOU A  
17 VERY BRIEF OVERVIEW OF THE TWO APPLICATIONS THAT  
18 WERE RECOMMENDED SO YOU HAVE A SENSE OF WHAT IT IS  
19 THAT WE ARE BRINGING TO FUND.

20 SO THE FIRST APPLICATION IS TRAN4-09884.  
21 THE TITLE IS "A NOVEL ROBUST AND COMPREHENSIVE  
22 PREDICTIVE TOOL USING HUMAN DISEASE-SPECIFIED  
23 INDUCED PLURIPOTENT STEM CELLS FOR CLINICAL DRUG  
24 SCREENING." THIS IS IN THE TOOL CATEGORY,  
25 OBVIOUSLY. THE GOAL HERE IS TO ADDRESS THE

1 CHALLENGE FOR DRUG DEVELOPERS, THAT MANY DRUG  
2 DEVELOPERS FIND THAT DRUGS ARE EITHER WITHDRAWN FROM  
3 THE MARKET OR FROM LATE STAGE DEVELOPMENT DUE TO  
4 UNPREDICTABLE CARDIOTOXICITY ONCE THESE DRUGS  
5 ACTUALLY GET TO PATIENTS OR TO CLINICAL TRIALS. AND  
6 THIS, THE APPLICANT FEELS, IS DUE LARGELY TO  
7 INEFFICIENT OR INEFFECTIVE SCREENING ASSAYS.

8 SO WHAT THEY PROPOSE TO DO HERE IS TO  
9 CREATE AN ASSAY THAT WOULD CIRCUMVENT THIS AND  
10 PREVENT THE COSTS AND THE EFFORT IN TAKING SUCH  
11 DRUGS THROUGH THAT DEVELOPMENT PROCESS. SO THEY  
12 HAVE CREATED AN INDUCED PLURIPOTENT STEM  
13 CELL-DERIVED CARDIOMYOCYTE SCREENING PLATFORM. THEY  
14 TAKE IPS CELLS FROM HEALTHY SUBJECTS AS WELL AS  
15 THOSE THAT HAVE COMMON HEREDITARY CARDIAC DISORDERS  
16 IN ORDER TO CREATE THAT SCREENING PLATFORM FOR SMALL  
17 MOLECULE DRUGS IN ORDER TO PREDICT WHETHER THESE  
18 COULD HAVE CARDIOTOXIC EFFECTS.

19 THIS APPLICATION SCORED A 90. THIRTEEN  
20 OUT OF 13 OF THE GWG REVIEWERS SCORED IT IN THE TOP  
21 TIER. AND JUST VERY BRIEFLY, THIS IS AN APPLICATION  
22 THAT THE REVIEWERS REALLY LOVED. THIS IS THE SECOND  
23 TIME THIS CAME AROUND, AND IT HAD A HIGH SCORE. IT  
24 HAD SOME ADMINISTRATIVE ISSUES THAT BROUGHT IT BACK  
25 THROUGH THE GWG. BUT EVEN AFTER THE SECOND REVIEW,

1 IT WAS CLEAR THAT THIS IS A STRONG PROPOSAL. IT WAS  
2 FELT THAT THE NEED TO DEVELOP THE TOOL THAT THEY  
3 PROPOSE COULD HAVE A GREAT IMPACT IN THE RESEARCH  
4 COMMUNITY AND IN DRUG DEVELOPMENT. THEY FEEL THE  
5 TEAM IS OUTSTANDING AND THAT THEY HAVE A  
6 COMPREHENSIVE STRATEGY FOR DEVELOPING THIS TOOL.

7 THERE WAS A CONCERN THAT REALLY RELATED TO  
8 THE MANUFACTURING AND DISTRIBUTION OF THIS PRODUCT,  
9 THAT THIS PLAN FOR DOING SO WAS NOT ADEQUATELY  
10 ADDRESSED IN THE APPLICATION. IT WASN'T A MAJOR  
11 CONCERN TO THEM AND SOMETHING THAT THEY FELT THAT  
12 THEY COULD ACCOMPLISH. THAT'S THE FIRST  
13 APPLICATION.

14 SHALL WE PAUSE AND CONSIDER IT OR SHOULD I  
15 GO THROUGH BOTH OF THEM?

16 SUPERVISOR SHEEHY: SINCE WE ONLY HAVE TWO  
17 IN THE FUNDABLE CATEGORY, LET'S DEAL WITH THOSE  
18 FIRST, AND WE'LL TAKE THEM ONE AT THAT TIME. IS  
19 THERE A MOTION TO FUND THIS APPLICATION?

20 CHAIRMAN THOMAS: SO MOVED.

21 SUPERVISOR SHEEHY: IS THERE A SECOND?

22 DR. PRIETO: SECOND.

23 SUPERVISOR SHEEHY: SECOND BY DR. PRIETO.  
24 ANY BOARD COMMENT? ANY PUBLIC COMMENT? MARIA,  
25 COULD YOU CALL THE ROLL PLEASE.



1 MS. BONNEVILLE: ANNE-MARIE DULIEGE.  
2 DAVID HIGGINS.  
3 DR. HIGGINS: YES.  
4 MS. BONNEVILLE: STEVE JUELSGAARD.  
5 DR. JUELSGAARD: YES.  
6 MS. BONNEVILLE: SHERRY LANSING. KATHY  
7 LAPORTE.  
8 MS. LAPORTE: YES.  
9 MS. BONNEVILLE: LAUREN MILLER.  
10 MS. MILLER: YES.  
11 MS. BONNEVILLE: ADRIANA PADILLA.  
12 DR. PADILLA: YES.  
13 MS. BONNEVILLE: JOE PANETTA.  
14 MR. PANETTA: YES.  
15 MS. BONNEVILLE: FRANCISCO PRIETO.  
16 DR. PRIETO: AYE.  
17 MS. BONNEVILLE: ROBERT QUINT.  
18 DR. QUINT: YES.  
19 MS. BONNEVILLE: AL ROWLETT.  
20 MR. ROWLETT: YES.  
21 MS. BONNEVILLE: JEFF SHEEHY.  
22 SUPERVISOR SHEEHY: YES.  
23 MS. BONNEVILLE: OS STEWARD.  
24 DR. STEWARD: YES.  
25 MS. BONNEVILLE: JONATHAN THOMAS.

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CHAIRMAN THOMAS: YES.

MS. BONNEVILLE: ART TORRES.

MR. TORRES: AYE.

MS. BONNEVILLE: DIANE WINOKUR.

MS. WINOKUR: YES.

MS. BONNEVILLE: MOTION CARRIES.

SUPERVISOR SHEEHY: THANK YOU. THE SECOND ONE PLEASE, DR. SAMBRANO.

DR. SAMBRANO: THE SECOND APPLICATION IN THE TOP TIER IS TRAN1-09814. THIS IS ENTITLED "PREDICTABLE PRO-REGENERATIVE SCAFFOLD FOR TREATING SYMPTOMATIC PERIPHERAL ARTERY DISEASE." THIS IS A THERAPY CANDIDATE. THE IDEA HERE IS TO TAKE WHAT IS A SCAFFOLD MATERIAL FROM PORCINE MUSCLE WHERE THEY TAKE THE EXTRACELLULAR MATRIX COMPONENTS AND COMBINE THEM INTO A HYDROGEL THAT'S INJECTABLE THAT CAN BE INTRODUCED INTO THE PATIENT MUSCLE THAT IS SUFFERING IN THE LIMB FROM PERIPHERAL ARTERY DISEASE.

THE GOAL IS THAT WHEN THIS HYDROGEL IS INJECTED, THAT IT GENERATES A SERIES OF RECRUITMENT OF BLOOD VESSELS AND DIFFERENTIATION OF MUSCLE STEM CELLS IN ORDER TO IMPROVE BLOOD FLOW AND THE MUSCLE FUNCTION.

THIS APPLICATION ALSO, LIKE THE FIRST, RECEIVED A 90. FOURTEEN OUT OF 14 MEMBERS OF THE

1 GRANTS WORKING GROUP SCORED IT IN THE TOP TIER.

2 REVIEWERS, BRIEFLY, FELT THAT WAS AN  
3 APPLICATION THAT PRESENTED A VERY STRONG PLAN. THEY  
4 HAVE GOOD PRECLINICAL DATA, AND THE PRECLINICAL  
5 STUDIES SHOWED INCREASED VASCULAR PERFUSION,  
6 INCREASED MUSCLE DEVELOPMENT AS A STRONG PROOF OF  
7 CONCEPT, AS WELL AS SAFETY. THIS IS AN APPLICATION  
8 THAT WAS RESUBMITTED, AND THE GWG MEMBERS FELT THE  
9 APPLICANT PROVIDED VERY GOOD RESPONSES TO THE  
10 PREVIOUS CONCERNS THAT THEY HAD.

11 THE ONE RECOMMENDATION, PERHAPS, AS  
12 OPPOSED TO MAYBE A CONCERN, THAT THE REVIEWERS FELT  
13 THAT THE APPLICANT SHOULD HAVE A PRE-IND MEETING  
14 WITH THE FDA SOONER RATHER THAN LATER IN ORDER TO  
15 MAKE SURE THAT THEY ALIGN THEIR IND PLAN PROPERLY  
16 AND CAN ENSURE THEY HAVE A GOOD PLAN GOING FORWARD  
17 FOR THIS PROJECT.

18 AND THEN THE SECOND CONCERN WAS TO FOCUS  
19 IN ON CRITICAL LIMB ISCHEMIA AS OPPOSED TO THE  
20 PATIENT POPULATION WHICH THEY ARE LARGELY TARGETING  
21 WHICH IS A PATIENT POPULATION THAT IS CALLED INDUCED  
22 CLAUDICATION. IT IS A PATIENT POPULATION THAT  
23 EXPERIENCES PAIN IN RESPONSE TO EXERCISE. SO IT IS  
24 A TYPE OF PERIPHERAL ARTERY DISEASE. THEY FELT THAT  
25 THE GREATEST NEED IS IN CLI AND WOULD LIKE TO SEE

1 THE APPLICANTS MOVE INTO THAT ARENA.

2 SUPERVISOR SHEEHY: THANK YOU, DR.

3 SAMBRANO.

4 CAN I GET A MOTION TO EITHER FUND OR NOT?

5 I GUESS A MOTION TO FUND WOULD BE APPROPRIATE.

6 DR. QUINT: SO MOVED.

7 MS. LAPORTE: SECOND.

8 SUPERVISOR SHEEHY: ARE THERE ANY BOARD

9 COMMENTS? ANY PUBLIC COMMENT?

10 DR. JUELSGAARD: JUST A QUICK QUESTION. I

11 NOTED IN THE COMMENTS UNDER CONCERNS THERE'S TALK

12 ABOUT A LEAD PRODUCT IN DEVELOPMENT. IT SOUNDS AS

13 IF THEY'VE GOT SOMETHING ELSE GOING ON IN ADDITION

14 TO OR BESIDES THIS; IS THAT RIGHT?

15 DR. SAMBRANO: THEY HAVE SOMETHING ELSE

16 THAT HAS BEEN IN THE CLINIC. AND SO PART OF THE

17 CONCERN THAT THE REVIEWERS EXPRESSED DURING THE

18 FIRST ONE IS THAT THEY WANTED TO HAVE A HEAD-TO-HEAD

19 COMPARISON WITH AN ANALOGOUS PRODUCT THAT THEY HAVE.

20 THIS IS AN IMPROVEMENT ON THAT PRODUCT. SO, YES,

21 THERE IS ANOTHER THAT THEY HAVE DEVELOPED THAT IS

22 SIMILAR.

23 DR. JUELSGAARD: ALL RIGHT. THANKS.

24 SUPERVISOR SHEEHY: ANY OTHER BOARD

25 COMMENT? MARIA, COULD YOU CALL THE ROLL PLEASE.

1 MS. BONNEVILLE: ANNE-MARIE DULIEGE.  
2 DAVID HIGGINS.  
3 DR. HIGGINS: YES.  
4 MS. BONNEVILLE: STEVE JUELSGAARD.  
5 DR. JUELSGAARD: YES.  
6 MS. BONNEVILLE: KATHY LAPORTE.  
7 MS. LAPORTE: YES.  
8 MS. BONNEVILLE: LAUREN MILLER.  
9 MS. MILLER: YES.  
10 MS. BONNEVILLE: ADRIANA PADILLA.  
11 DR. PADILLA: YES.  
12 MS. BONNEVILLE: JOE PANETTA.  
13 MR. PANETTA: YES.  
14 MS. BONNEVILLE: FRANCISCO PRIETO.  
15 DR. PRIETO: AYE.  
16 MS. BONNEVILLE: ROBERT QUINT.  
17 DR. QUINT: YES.  
18 MS. BONNEVILLE: AL ROWLETT.  
19 MR. ROWLETT: YES.  
20 MS. BONNEVILLE: JEFF SHEEHY.  
21 SUPERVISOR SHEEHY: YES.  
22 MS. BONNEVILLE: OS STEWARD.  
23 DR. STEWARD: YES.  
24 MS. BONNEVILLE: JONATHAN THOMAS.  
25 CHAIRMAN THOMAS: YES.

1 MS. BONNEVILLE: ART TORRES.

2 MR. TORRES: AYE.

3 MS. BONNEVILLE: DIANE WINOKUR.

4 MS. WINOKUR: YES.

5 MS. BONNEVILLE: MOTION CARRIES.

6 SUPERVISOR SHEEHY: THANK YOU.

7 SO NOW WE HAVE REMAINING ITEMS. ARE THERE  
8 ANY OTHER OF THE REMAINING ITEMS THAT ANYONE WOULD  
9 LIKE TO MOVE INTO THE FUNDABLE CATEGORY?

10 THEN COULD I GET A MOTION TO NOT FUND THE  
11 REMAINING ITEMS?

12 MR. ROWLETT: SO MOVED.

13 DR. JUELSGAARD: SECOND.

14 SUPERVISOR SHEEHY: ANY BOARD COMMENT?  
15 ANY PUBLIC COMMENT?

16 CHAIRMAN THOMAS: YES. WE HAVE DR. CHIU  
17 HERE AT CITY OF HOPE WHO WOULD LIKE TO GIVE PUBLIC  
18 COMMENT.

19 DR. CHIU: SO IT'S IN THE NATURE OF A  
20 QUESTION. CAN EVERYBODY HEAR ME?

21 MS. BONNEVILLE: YES.

22 DR. CHIU: ACCORDING TO DR. SAMBRANO'S  
23 CHART, THERE ARE NINE APPLICATIONS THAT HAD A SCORE  
24 OF 1 TO 84; BUT IN THE PRINTOUT, I NOTICED SIX IN  
25 THE GRAY ZONE. AND AM I MISREADING SOMETHING? WERE

1 THERE ONLY SIX THAT WERE SCORED 1 TO 84 OR WERE  
2 THERE NINE?

3 DR. SAMBRANO: THERE WERE 11 APPLICATIONS  
4 THAT WERE REVIEWED. TWO WERE RECOMMENDED, NINE WERE  
5 NOT, AND THE ONES THAT ARE NOT SHOWN ON THE  
6 SPREADSHEET WITHDREW BEFORE THE BOARD MEETING.

7 DR. CHIU: THANK YOU. AND A SECOND  
8 FOLLOW-UP QUICK QUESTION. THESE ARE THE ONES THAT  
9 WERE REVIEWED, THE TOTAL NINE PLUS TWO THAT YOU  
10 INDICATED. HOW MANY PROPOSALS CAME IN OUT OF WHICH  
11 THESE 11 WERE SELECTED FOR REVIEW?

12 DR. SAMBRANO: SO ALL OF THE ELEVEN  
13 APPLICATIONS THAT WERE REVIEWED WERE ALL THE  
14 ELIGIBLE APPLICATIONS THAT WERE RECEIVED.

15 DR. CHIU: POSITIVELY SELECTED, RIGHT?

16 DR. SAMBRANO: THERE WAS NO POSITIVE  
17 SELECTION.

18 DR. CHIU: THERE WAS NO POSITIVE  
19 SELECTION. THESE ARE ALL THAT MET ELIGIBILITY?

20 DR. SAMBRANO: CORRECT.

21 DR. CHIU: JUST CLARIFY FOR ME, IF YOU  
22 DON'T MIND, ARE TRAN PROPOSALS POSITIVELY SELECTED  
23 OR JUST BY ELIGIBILITY?

24 DR. SAMBRANO: IT DEPENDS ON HOW MANY  
25 PROPOSALS ARE RECEIVED. IF THERE ARE MORE PROPOSALS

1 THAN CAN BE TAKEN TO A SINGLE OR THROUGH A SINGLE  
2 CYCLE OF THE GWG, THEN THEY UNDERGO POSITIVE  
3 SELECTION. BUT SO FAR IN ALL THE TRAN CYCLES THAT  
4 WE'VE HAD, WE HAVE RECEIVED A NUMBER OF APPLICATIONS  
5 THAT MEANS WE DON'T HAVE TO CONDUCT A POSITIVE  
6 SELECTION. THEY HAVE ALL BEEN UNDER THE AMOUNT THAT  
7 WE CAN MANAGE.

8 DR. CHIU: OKAY. THANK YOU VERY MUCH.

9 SUPERVISOR SHEEHY: DO WE HAVE ANY OTHER  
10 PUBLIC COMMENT? MARIA, COULD YOU CALL THE ROLL  
11 PLEASE.

12 MR. HARRISON: JUST A REMINDER TO MEMBERS  
13 WHO MAY HAVE AN INTEREST IN AN APPLICATION THAT  
14 FALLS WITHIN THE TIERS, TO VOTE YES OR NO WITH  
15 RESPECT TO THOSE APPLICATIONS IN WHICH YOU HAVE A  
16 CONFLICT.

17 DR. JUELSGAARD: JEFF, COULD YOU REPEAT  
18 THE MOTION SO WE'RE JUST CLEAR ON WHAT WE'RE VOTING  
19 ON?

20 SUPERVISOR SHEEHY: THE MOTION IS TO NOT  
21 FUND ANY OF THE REMAINING APPLICATIONS, WHICH I  
22 THINK THERE'S SIX ON THIS PAGE.

23 MS. BONNEVILLE: ANNE-MARIE DULIEGE.  
24 DAVID HIGGINS.

25 DR. HIGGINS: YES.



1 MS. BONNEVILLE: STEVE JUELSGAARD.  
2 DR. JUELSGAARD: YES.  
3 MS. BONNEVILLE: SHERRY LANSING. KATHY  
4 LAPORTE.  
5 MS. LAPORTE: YES.  
6 MS. BONNEVILLE: LAUREN MILLER.  
7 MS. MILLER: YES.  
8 MS. BONNEVILLE: ADRIANA PADILLA.  
9 DR. PADILLA: YES.  
10 MS. BONNEVILLE: JOE PANETTA.  
11 MR. PANETTA: YES.  
12 MS. BONNEVILLE: FRANCISCO PRIETO.  
13 DR. PRIETO: AYE.  
14 MS. BONNEVILLE: ROBERT QUINT.  
15 DR. QUINT: YES.  
16 MS. BONNEVILLE: AL ROWLETT.  
17 MR. ROWLETT: YES.  
18 MS. BONNEVILLE: JEFF SHEEHY.  
19 SUPERVISOR SHEEHY: YES.  
20 MS. BONNEVILLE: OS STEWARD.  
21 DR. STEWARD: YES.  
22 MR. HARRISON: EXCEPT FOR THOSE WITH WHICH  
23 YOU HAVE A CONFLICT.  
24 DR. STEWARD: EXCEPT FOR THOSE WITH WHICH  
25 I HAVE A CONFLICT. MY APOLOGIES.

1 MS. BONNEVILLE: JONATHAN THOMAS.

2 CHAIRMAN THOMAS: YES.

3 MS. BONNEVILLE: EXCEPT FOR THOSE WITH  
4 WHICH YOU HAVE A CONFLICT.

5 CHAIRMAN THOMAS: EXCEPT FOR THOSE WITH  
6 WHICH I HAVE A CONFLICT.

7 MS. BONNEVILLE: ART TORRES.

8 MR. TORRES: AYE.

9 MS. BONNEVILLE: DIANE WINOKUR.

10 MS. WINOKUR: YES.

11 MS. BONNEVILLE: THANK YOU. THE MOTION  
12 CARRIES.

13 SUPERVISOR SHEEHY: I THINK THAT'S IT FOR  
14 BUSINESS TODAY. IT'S BACK TO YOU IF YOU WANT TO  
15 CLOSE OUT THE MEETING, CHAIRMAN THOMAS.

16 CHAIRMAN THOMAS: THANK YOU, SUPERVISOR  
17 SHEEHY.

18 LAST THING ON THE AGENDA IS GENERAL PUBLIC  
19 COMMENT ON ANY ITEMS OF INTEREST. HEARING NONE, WE  
20 STAND ADJOURNED. HAVE A GOOD DAY, EVERYBODY.

21 (THE MEETING WAS THEN CONCLUDED AT  
22 11:37 A.M.)

23

24

25

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 OF THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE AND THE APPLICATION REVIEW SUBMITTEE IN THE MATTER OF ITS REGULAR MEETING ON MARCH 23, 2017, 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, CA CSR 7152  
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