

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: MAY 25, 2017
11 A.M.

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

FILE NO.: 2017-13

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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 CLIN 1: PARTNERING OPPORTUNITY FOR LATE STAGE PRECLINICAL PROJECTS.	4
4. CONSIDERATION OF APPLICATIONS SUBMITTED IN RESPONSE TO THE DISC1: INCEPTION AWARDS.	8
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 CLIN 1: PARTNERING OPPORTUNITY FOR LATE STAGE PRECLINICAL PROJECTS AND THE DISC1: INCEPTION AWARDS (HEALTH & SAFETY CODE 125290.30(F) (3) (B) AND (C)).	
6. PUBLIC COMMENT.	NONE
7. ADJOURNMENT.	19

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MAY 25, 2017; 11 A.M.

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

MS. BONNEVILLE: GEORGE BLUMENTHAL. DAVID BRENNER. KEN BURTIS. DEBORAH DIES. ANNE-MARIE DULIEGE.

DR. DULIEGE: PRESENT.

MS. BONNEVILLE: HOWARD FEDEROFF. JUDY GASSON. SAM HAWGOOD. DAVID HIGGINS.

DR. HIGGINS: YES, HERE.

MS. BONNEVILLE: STEVE JUELSGAARD.

DR. JUELSGAARD: HERE.

MS. BONNEVILLE: SHERRY LANSING. KATHY LAPORTE. SHLOMO MELMED. LAUREN MILLER.

MS. MILLER: HERE.

MS. BONNEVILLE: LLOYD MINOR. ADRIANA PADI LLA.

DR. PADI LLA: HERE.

MS. BONNEVILLE: JOE PANETTA.

MR. PANETTA: I'M HERE. AND I JUST WANT TO MENTION THAT I DO HAVE A MEMBER OF THE PUBLIC HERE FOR THE PUBLIC COMMENT PERIOD.

1 MS. BONNEVILLE: THANK YOU.
2 FRANCISCO PRIETO.
3 DR. PRIETO: HERE.
4 MS. BONNEVILLE: CARMEN PULIAFITO. ROBERT
5 QUINT. AL ROWLETT.
6 MR. ROWLETT: HERE.
7 MS. BONNEVILLE: JEFF SHEEHY.
8 SUPERVISOR SHEEHY: HERE.
9 MS. BONNEVILLE: OS STEWARD.
10 DR. STEWARD: HERE.
11 MS. BONNEVILLE: JONATHAN THOMAS.
12 CHAIRMAN THOMAS: HERE.
13 MS. BONNEVILLE: ART TORRES.
14 MR. TORRES: HERE.
15 MS. BONNEVILLE: KRISTINA VUORI. DIANE
16 WINOKUR.
17 WE'LL FOLLOW UP WITH DIANE AND ROBERT
18 QUINT. WE HAVE THEM AS --
19 DR. QUINT: ROBERT QUINT, HERE.
20 MS. BONNEVILLE: GREAT. THANKS,
21 DR. QUINT.
22 WE HAVE A QUORUM.
23 CHAIRMAN THOMAS: THANK YOU, MARIA.
24 WE'LL MOVE ON TO ITEM 3, CONSIDERATION OF
25 APPLICATIONS PRESENTED IN RESPONSE TO CLIN1,

1 PARTNERING OPPORTUNITY FOR LATE STAGE PRECLINICAL
2 PROJECTS. I WILL NOW TURN THE MEETING OVER TO
3 SUPERVISOR SHEEHY.

4 SUPERVISOR SHEEHY: THANKS, J. T. GIL, DO
5 YOU HAVE A PRESENTATION?

6 DR. SAMBRANO: YES, I DO? SHALL I GO
7 AHEAD?

8 SUPERVISOR SHEEHY: YES, PLEASE.

9 DR. SAMBRANO: THANK YOU. SO GOOD
10 MORNING, EVERYONE. THIS MORNING I'M GOING TO
11 PRESENT A PROPOSAL THAT COMES TO US RESPONDING TO
12 THE CLINICAL STAGE PROGRAM CALLED SPECIFICALLY THE
13 CLIN1 THAT PROVIDES FUNDING FOR PROJECTS THAT ARE
14 DOING IND-ENABLING STUDIES THAT INTEND TO GET THEM
15 TOWARDS A CLINICAL TRIAL.

16 JUST A REMINDER OF THE SCORING SYSTEM SO
17 THAT WE'RE ALL ON THE SAME PAGE. WE HAVE A SCORING
18 SYSTEM OF 1, 2, AND 3, WITH A SCORE OF 1 MEANING
19 EXCEPTIONAL MERIT; A SCORE OF 2 MEANS THAT THE
20 APPLICATION NEEDS IMPROVEMENT AND DOES NOT WARRANT
21 FUNDING AT THIS TIME, BUT CAN BE RESUBMITTED TO
22 IMPROVE THOSE AREAS OF CONCERN; AND A THEN SCORE OF
23 3, WHICH MEANS IT'S SUFFICIENTLY FLAWED THAT IT
24 DOESN'T WARRANT FUNDING AND THEY SHOULD NOT SUBMIT
25 THE APPLICATION FOR AT LEAST SIX MONTHS.

1 SO THE APPLICATION UNDER CONSIDERATION IS
2 CLIN1-09811. THIS IS A CLINICAL DEVELOPMENT PROJECT
3 FOCUSED ON A CELL THERAPY OR DEVELOPING A CELL
4 THERAPY FOR STROKE. THE INTENDED THERAPEUTIC IS A
5 MESENCHYMAL STROMAL CELLS, AND THE INDICATION IS FOR
6 ACUTE ISCHEMIC STROKE.

7 AND SO THEIR GOAL IS TO COMPLETE
8 PRECLINICAL ACTIVITIES TO FILE AN IND FOR TESTING
9 THE CELL THERAPY PRODUCT AND, MORE SPECIFICALLY, ARE
10 ACTIVITIES THAT THE FDA HAS SUGGESTED THAT THEY DO
11 PRIOR TO THEIR FILING. SO THEY HAVE KIND OF AN
12 ITEMIZED LIST OF ACTIVITIES THAT WERE REQUESTED.
13 AND SO THAT'S WHAT THEY INTEND TO DO, AND THEY ARE
14 REQUESTING 1.38 MILLION IN ORDER TO DO THOSE
15 STUDIES.

16 ON THE LAST SLIDE THERE IS AN OVERVIEW OF
17 THE OUTCOME OF THE REVIEW. AS I MENTIONED IN
18 PREVIOUS TIMES, WE UNDERGO A BUDGET REVIEW WITH EACH
19 OF THE APPLICATIONS. NO ISSUES WERE HIGHLIGHTED IN
20 REGARDS TO THE BUDGET. HOWEVER, WITH THE GRANTS
21 WORKING GROUP REVIEW, THE GRANTS WORKING GROUP
22 REVIEWED THIS AND GAVE IT A SCORE OF 3, WHICH THEN
23 MEANS DOES NOT WARRANT FUNDING, WITH NO MEMBERS
24 GIVING IT A SCORE OF 1, THERE WERE TWO MEMBERS WHO
25 GAVE IT A SCORE OF 2, AND 13 THAT GAVE IT A SCORE OF

1 THREE.

2 AND THE CIRM TEAM ALSO REVIEWS THESE
3 RECOMMENDATIONS FROM A PROCESS PERSPECTIVE AND WE
4 ARE IN CONCURRENCE WITH NOT FUNDING THIS PARTICULAR
5 APPLICATION.

6 SO, MR. SHEEHY, BACK TO YOU.

7 SUPERVISOR SHEEHY: SO DO I HAVE A MOTION
8 TO ACCEPT THE GRANTS WORKING GROUP AND THE CIRM
9 TEAM'S RECOMMENDATION THAT WE DO NOT FUND THIS
10 APPLICATION?

11 DR. DULIEGE: I CAN MAKE THIS MOTION.
12 YES. I AGREE WITH THIS MOTION.

13 DR. PRIETO: I'LL SECOND.

14 SUPERVISOR SHEEHY: OKAY. WE'VE GOT A
15 MOTION AND A SECOND. IS THERE ANY DISCUSSION
16 AMONGST BOARD MEMBERS? ANY PUBLIC COMMENT? SO
17 COULD WE CALL THE ROLL THEN?

18 MS. BONNEVILLE: YES.

19 ANNE-MARIE DULIEGE.

20 DR. DULIEGE: YES.

21 MS. BONNEVILLE: DAVID HIGGINS.

22 DR. HIGGINS: YES.

23 MS. BONNEVILLE: STEVE JUELGAARD.

24 DR. JUELGAARD: YES.

25 MS. BONNEVILLE: KATHY LAPORTE. LAUREN

1 MILLER.
2 MS. MILLER: YES.
3 MS. BONNEVILLE: ADRIANA PADILLA.
4 DR. PADILLA: YES.
5 MS. BONNEVILLE: JOE PANETTA.
6 MR. PANETTA: YES.
7 MS. BONNEVILLE: FRANCISCO PRIETO.
8 DR. PRIETO: AYE.
9 MS. BONNEVILLE: ROBERT QUINT.
10 DR. QUINT: YES.
11 MS. BONNEVILLE: AL ROWLETT.
12 MR. ROWLETT: YES.
13 MS. BONNEVILLE: JEFF SHEEHY.
14 SUPERVISOR SHEEHY: YES.
15 MS. BONNEVILLE: JONATHAN THOMAS.
16 CHAIRMAN THOMAS: YES.
17 MS. BONNEVILLE: ART TORRES.
18 MR. TORRES: AYE.
19 MS. BONNEVILLE: DIANE WINOKUR.
20 MOTION CARRIES.
21 SUPERVISOR SHEEHY: THANK YOU. SO GOING
22 TO THE NEXT ITEM, CONSIDERATION OF INCEPTION OF THE
23 DISC1 INCEPTION AWARDS. GIL, DO WE HAVE A
24 PRESENTATION FOR THAT?
25 DR. SAMBRANO: YES, I DO.

1 SO ON THE WEBEX YOU HAVE EITHER SLIDES
2 THAT WERE PROVIDED TO YOU, YOU CAN FOLLOW THERE, OR
3 ON WEBEX IF YOU HAVE IT. IF ABSOLUTELY NECESSARY,
4 I WILL PRETTY MUCH TELL YOU EVERYTHING THAT'S ON
5 THESE SLIDES.

6 THE FIRST ONE IS JUST AN ILLUSTRATION THAT
7 SHOWS THE TRACKS OF OUR CONTINUOUS AND RECURRING
8 FUNDING OPPORTUNITIES FROM DISCOVERY THROUGH THE
9 CLINIC. AND THE POINT OF THIS IS JUST TO HELP PLACE
10 THE INCEPTION PROGRAM IN YOUR MIND. IT IS REALLY AT
11 THE VERY BEGINNING OF DISCOVERY. IT IS A SEED
12 PROGRAM, SO IT IS THE LAUNCHING OF AN IDEA. AND
13 THAT'S WHERE WE ARE CREATING THIS PARTICULAR FUNDING
14 OPPORTUNITY.

15 AND THAT THE OBJECTIVE IS TO PROVIDE SEED
16 FUNDING FOR WHAT ARE HOPEFULLY GREAT IDEAS THAT WILL
17 HAVE AN IMPACT ON THE FIELD OF HUMAN STEM CELL
18 RESEARCH, BUT WHERE THEY MAY NEED SOME MODEST
19 SUPPORT TO ACTUALLY TEST THE IDEA, SEE IF IT WORKS,
20 AND THEN COMPETE FOR LARGER FUNDING OPPORTUNITIES.

21 AS SUCH, THE EMPHASIS OF THE REVIEW AND
22 OUR INSTRUCTIONS TO THE GRANTS WORKING GROUP REVIEW
23 MEMBERS WAS THAT WE ARE LOOKING FOR GREAT NEW IDEAS.
24 SO THAT'S WHERE WE NEED THEIR HELP IN IDENTIFYING
25 THEM, THOSE THAT HAVE THE POTENTIAL TO ULTIMATELY

1 RESULT IN SOMETHING THAT MAY BE A TRANSLATABLE HUMAN
2 STEM PROGENITOR-BASED PRODUCT OR TOOL.

3 WE LIKE IDEAS THAT HAVE A SOUND SCIENTIFIC
4 RATIONALE. AND, OF COURSE, THAT IS IMPORTANT, BUT
5 WE ALSO EMPHASIZE THAT PRELIMINARY DATA IS NOT
6 REQUIRED OR EXPECTED AT THIS STAGE. WE WANT TO
7 FOCUS THE REVIEW ON WHETHER THE IMPACT CAN BE GREAT
8 AND ALLOW FOLKS TO GIVE SOMETHING THAT HAS SOME
9 POTENTIAL FOR IMPACT A SHOT. AND, THUS, THIS IS A
10 HIGH RISK, HIGH REWARD PROGRAM.

11 WE WILL PROVIDE 150 K TO TEST THE IDEA, SO
12 IT IS A MODEST AMOUNT TO GENERATE THE DATA. SO
13 THOSE WERE GENERAL INSTRUCTIONS TO REVIEWERS AS THEY
14 WENT INTO THE REVIEW, UTILIZE THE SELECTED
15 PROPOSALS.

16 WE STARTED WITH A POSITIVE SELECTION
17 PROCESS. SO WE HAD 72 PROPOSALS THAT WE STARTED
18 WITH. AND THROUGH THE POSITIVE SELECTION PROCESS,
19 THE PANEL ITSELF THAT REVIEWED THEM THROUGHOUT THE
20 COURSE WERE THE SAME ONES THAT DID THE POSITIVE
21 SELECTION AS WELL AS THE FINAL REVIEW. THEY
22 NARROWED THE APPLICATIONS DOWN TO 38 PROPOSALS.
23 AND, OF COURSE, SELECTION BY ANY ONE MEMBER OF THE
24 GWG, EITHER SCIENTIFIC OR PATIENT ADVOCATE MEMBER,
25 WAS SUFFICIENT TO MOVE AN APPLICATION FORWARD.

1 THE SCORING SYSTEM THAT WE USE IN
2 DISCOVERY AND TRANSLATION, JUST, AGAIN, AS A
3 REMINDER BECAUSE IT IS DIFFERENT FROM CLINICAL, WE
4 USE A SCALE OF ONE TO A HUNDRED, AND THE MEDIAN OF
5 ALL THE SCORING REVIEWERS IS WHAT YOU UTILIZE AS THE
6 SCORE. APPLICATIONS THAT SCORE BETWEEN 85 AND 100
7 ARE DEEMED TO HAVE EXCEPTIONAL MERIT AND WARRANT
8 FUNDING. THOSE THAT RECEIVE A SCORE FROM 1 TO 84
9 ARE NOT RECOMMENDED FOR FUNDING.

10 AND FOR THIS ROUND OF THE DISCOVERY¹ OR
11 THE INCEPTION PROGRAM, THE SUMMARY OF THE
12 RECOMMENDATIONS ARE SHOWN ON THE TABLE WHERE WE HAD
13 SIX APPLICATIONS THAT WERE IN THE TOP FUNDING
14 CATEGORY, WHICH WOULD TOTAL TO ABOUT 1.37 MILLION IN
15 REQUESTED FUNDS FROM THOSE SIX APPLICATIONS, AND
16 THEN 32 OTHERS THAT WERE NOT SELECTED.

17 I HAVE A SLIDE HERE JUST TO REMIND YOU
18 THAT, AT THE CONCLUSION OF EACH OF THE REVIEWS, THE
19 GRANTS WORKING GROUP TAKES A VOTE ON THE REVIEW
20 PROCESS. AND IN THIS CASE THE PATIENT ADVOCATE
21 MEMBERS AND SCIENTIFIC MEMBERS UNANIMOUSLY FOUND THE
22 APPROPRIATENESS OF THE PROCESS UNDERTAKEN FOR THIS
23 REVIEW.

24 BEFORE TURNING IT OVER, I MIGHT JUST VERY
25 BRIEFLY GO OVER EACH OF THE SIX APPLICATIONS THAT

1 WERE RECOMMENDED FOR FUNDING JUST TO GIVE YOU A HIGH
2 LEVEL VIEW OF WHAT THEY ARE. I'LL BE BRIEF SINCE I
3 KNOW WE DON'T HAVE THAT MUCH TIME TODAY.

4 SO THE FIRST APPLICATION IS 10074. IT'S
5 ENTITLED "REPROGRAMMING HUMAN STEM CELLS FOR BLOOD
6 CELL GENERATION." THE GOAL OF THIS APPLICATION IS
7 TO CREATE A UNIVERSAL DONOR BLOOD CELL LINE; THAT
8 IS, TO CREATE THE ABILITY TO GENERATE RED BLOOD
9 CELLS FOR TRANSPLANTATION. AND THIS APPLICATION
10 RECEIVED THE TOP SCORE OF 90 WITH ALL SCIENTIFIC
11 MEMBERS UNANIMOUSLY VOTING IN FAVOR OF FUNDING THIS
12 APPLICATION.

13 THE NEXT ONE IN LINE IS 10036. THIS ONE
14 IS ENTITLED "PRO-DRUG INNOVATION TO TARGET MUSCLE
15 STEM CELLS AND ENHANCE MUSCLE REGENERATION." THE
16 OBJECTIVE IN THIS APPLICATION IS TO DEVELOP AN
17 APPROACH TO TARGET MUSCLE STEM CELLS OR THERAPEUTICS
18 TO MUSCLE STEM CELLS. AND THE IDEA HERE IS THAT
19 THEY WILL GENERATE EITHER A PEPTIDE OR A FRAGMENT OF
20 THE ANTIBODY THAT RECOGNIZES A NEW PROTEIN THAT WAS
21 IDENTIFIED IN THESE MUSCLE STEM CELLS IN ORDER TO
22 DIRECT THERAPEUTICS TO THOSE MUSCLE STEM CELLS.

23 THIS RECEIVED A SCORE OF 86, AGAIN,
24 UNANIMOUSLY BY THE GROUP.

25 THE THIRD APPLICATION IS 09912. THIS ONE

1 IS ENTITLED "NOVEL TISSUE ENGINEERING TECHNIQUE TO
2 REPAIR DEGENERATED RETINA." THE OBJECTIVE HERE IS
3 TO DEVELOP A THERAPEUTIC PRODUCT FROM HUMAN
4 EMBRYONIC STEM CELLS TO TREAT ADVANCED HUMAN RETINA
5 DEGENERATIVE DISEASES. AND THIS COMBINES WHAT'S
6 RETINA ORGANOIDS ALONG WITH RETINAL PIGMENT
7 EPI THELIUM COMBINED IN ORDER TO REPAIR DAMAGED OR
8 DISEASED EYES.

9 THIS APPLICATION RECEIVED A SCORE OF 85,
10 AGAIN, A FAVORABLE RECOMMENDATION UNANIMOUSLY BY THE
11 GROUP.

12 THE FOURTH APPLICATION IS 10079 ENTITLED
13 "AN EXOSOME-BASED TRANSLATIONAL STRATEGY TO MITIGATE
14 ALZHEIMER'S DISEASE NEUROPATHOLOGY." THE GOAL HERE
15 IS TO DEVELOP A THERAPEUTIC PRODUCT FROM STEM
16 CELL-DERIVED EXOSOMES, MORE SPECIFICALLY, NEURAL
17 STEM CELLS WHICH RELEASE THESE EXOSOMES, AND THEY
18 ISOLATE THEM IN ORDER TO TREAT ALZHEIMER'S DISEASE
19 SYMPTOMS.

20 THIS APPLICATION RECEIVED ALSO AN 85 AND
21 WAS UNANIMOUSLY RECOMMENDED.

22 THE FIFTH APPLICATION IS 09984. THE TITLE
23 IS "HYPO-IMMUNOGENIC CARDIAC PATCHES FOR MYOCARDIAL
24 REGENERATION." THE GOAL OF THIS PROPOSAL IS TO
25 ENGINEER AN ALLOGENEIC CARDIAC PATCH THAT WOULD

1 RESTORE FUNCTION AFTER A HEART ATTACK, BUT THAT DOES
2 NOT ELICIT AN IMMUNE RESPONSE. SO BY CREATING A
3 CELL LINE THAT IS MISSING THE MAJOR
4 HISTOCOMPATIBILITY PROTEINS, THEY HOPE TO CREATE A
5 LINE THAT CAN BE USED WITHOUT WORRY OF IMMUNE
6 ISSUES.

7 THIS APPLICATION RECEIVED A SCORE OF 85,
8 AND 13 OUT OF 14 MEMBERS GAVE IT A RECOMMENDATION
9 FOR FUNDING.

10 THE LAST APPLICATION THAT IS IN THE
11 RECOMMENDED TIER IS 09999. IT'S ENTITLED
12 "GENERATION OF EXPANDABLE SELF-RENEWING MUSCLE STEM
13 CELLS FOR DUCHENNE MUSCULAR DYSTROPHY." AND THE
14 OBJECTIVE OF THIS IS TO GENERATE EXPANDABLE
15 SELF-RENEWING HUMAN MUSCLE STEM CELLS, HUMAN INDUCED
16 PLURIPOTENT STEM CELLS, IN ORDER TO CREATE A MODEL
17 FOR STUDYING DUCHENNE MUSCULAR DYSTROPHY AND PERHAPS
18 THEN DEVELOP ULTIMATELY A THERAPEUTIC FOR IT.

19 THIS APPLICATION SCORED AN 85 WITH NINE
20 MEMBERS GIVING IT A SCORE OF RECOMMENDED AND FIVE
21 WHO DID NOT.

22 AND SO THAT IS A SUMMARY OF THE
23 APPLICATIONS THAT ARE RECOMMENDED. HAPPY TO TAKE
24 QUESTIONS. MR. SHEEHY.

25 SUPERVISOR SHEEHY: ANY QUESTIONS FROM

1 MEMBERS OF THE BOARD? SO IS THERE A MOTION TO MOVE
2 ANY APPLICATION FROM TIER I TO TIER II? IS THERE A
3 MOTION TO MOVE ANY APPLICATION FROM TIER II INTO
4 TIER I? SO COULD I GET A MOTION TO FUND ALL THE
5 APPLICATIONS IN TIER I AND TO NOT FUND THE
6 APPLICATIONS IN TIER II?

7 CHAIRMAN THOMAS: SO MOVED.

8 SUPERVISOR SHEEHY: MARIA, DID YOU CATCH
9 WHO MADE THE MOTION AND WHO SECONDED?

10 MS. BONNEVILLE: NO. IT WOULD BE GREAT IF
11 WE COULD CLARIFY.

12 CHAIRMAN THOMAS: J. T. MADE THE MOTION.

13 MR. PANETTA: SECOND.

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

16 MR. PANETTA: WE'VE GOT PUBLIC COMMENT
17 HERE. THIS IS JOE.

18 MS. BONNEVILLE: GREAT.

19 DR. SACCO: (UNINTELLIGIBLE.)

20 THE REPORTER: I'M SORRY, MR. CHAIRMAN, I
21 CAN'T UNDERSTAND THE SPEAKER.

22 MS. BONNEVILLE: PLEASE SPEAK DIRECTLY
23 INTO THE PHONE.

24 DR. SACCO: IS IT BETTER NOW?

25 THE REPORTER: YES. THANK YOU.

1 DR. SACCO: SO I WAS JUST SAYING THAT SO
2 THE GOAL OF THIS APPLICATION IS TO OVERCOME THE
3 BOTTLENECK OF GENERATING EXPANDABLE MUSCLE STEM
4 CELLS FROM HUMAN IPS CELLS. SO FAR ALL THE
5 APPROACHES HAVE BEEN GENERATING COMMITTED
6 PROGENITORS THAT ACTUALLY HAVE LIMITED CELL
7 SELF-RENEWAL, SO THEY ARE NEEDED IN THEIR ABILITY TO
8 BEING USED FOR MODELING IN VITRO AND ALSO IN
9 THERAPIES. SO THE APPROACH THAT WE ARE PROPOSING IS
10 TO COMBINE PROTOCOLS THAT RECAPITULATE TOGETHER WITH
11 THE USE OF OTHER INHIBITORS WHICH WE HAVE RECENTLY
12 SHOWN ARE IMPORTANT IN MUSCLE TO EXPAND MUSCLE STEM
13 CELLS. SO REALLY THE GOAL IS TO INTEGRATE THESE TWO
14 ASSETS AND TO ACTUALLY TEST MUSCLE STEM CELL
15 GENERATED BOTH IN VITRO AND IN VIVO TRANSPLANTATION
16 ASSAY (UNINTELLIGIBLE).

17 AND SO, OF COURSE, WITH THE APPLICATION
18 NOT ONLY FOR DUCHENNE MUSCULAR DYSTROPHY, WHICH IS
19 THE FOCUS OF THE APPLICATION, BUT ALSO MORE BROADLY
20 TO OTHER MUSCLE DISEASES BECAUSE IT WOULD ENABLE US
21 TO RECAPITULATE (UNINTELLIGIBLE) OF MYOGENESIS IN
22 OTHER HUMAN DISEASES. I JUST WANTED TO ADD THAT.
23 THANK YOU.

24 SUPERVISOR SHEEHY: THANK YOU. DO WE HAVE
25 ANOTHER PUBLIC COMMENT? SO, MARIA, COULD YOU CALL

1 THE ROLL PLEASE?

2 MR. HARRISON: JEFF, IT'S JAMES. JUST A
3 REMINDER TO THOSE MEMBERS WHO HAVE A CONFLICT WITH
4 RESPECT TO ANY APPLICATION IN TIER I OR TIER II TO
5 VOTE YES OR NO FOR THE MOTION EXCEPT FOR THOSE
6 APPLICATIONS IN WHICH YOU HAVE A CONFLICT.

7 MS. BONNEVILLE: ANNE-MARIE DULIEGE.

8 DR. DULIEGE: YES.

9 MS. BONNEVILLE: DAVID HIGGINS.

10 DR. HIGGINS: YES.

11 MS. BONNEVILLE: STEVE JUELGAARD.

12 DR. JUELGAARD: YES.

13 MS. BONNEVILLE: SHERRY LANSING. KATHY
14 LAPORTE. LAUREN MILLER.

15 MS. MILLER: YES.

16 MS. BONNEVILLE: ADRIANA PADI LLA.

17 DR. PADI LLA: YES.

18 MS. BONNEVILLE: JOE PANETTA.

19 MR. PANETTA: YES.

20 MS. BONNEVILLE: FRANCISCO PRIETO.

21 DR. PRIETO: AYE.

22 MS. BONNEVILLE: ROBERT QUINT.

23 DR. QUINT: YES.

24 MS. BONNEVILLE: AL ROWLETT.

25 MR. ROWLETT: YES.

1 MS. BONNEVILLE: JEFF SHEEHY.

2 SUPERVISOR SHEEHY: YES.

3 MS. BONNEVILLE: OS STEWARD.

4 DR. STEWARD: YES EXCEPT FOR THOSE WITH
5 WHICH I'M IN CONFLICT.

6 MS. BONNEVILLE: JONATHAN THOMAS. ART
7 TORRES.

8 MR. TORRES: AYE.

9 CHAIRMAN THOMAS: SORRY. I HAD IT ON
10 MUTE, MARIA. YES.

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

13 SUPERVISOR SHEEHY: GREAT. THANK YOU,
14 MARIA.

15 SO IT'S BACK TO YOU, J.T., AND I THINK
16 WE'VE CONCLUDED THE BUSINESS OF THE APPLICATION
17 REVIEW SUBCOMMITTEE.

18 CHAIRMAN THOMAS: THANK YOU, MR.
19 SUPERVISOR.

20 LAST ITEM ON THE AGENDA IS GENERAL PUBLIC
21 COMMENT. DO WE HAVE ANY SUCH COMMENTS? HEARING
22 NONE, THAT CONCLUDES TODAY'S MEETING. DO I HEAR A
23 MOTION TO ADJOURN?

24 MR. TORRES: SO MOVED.

25 CHAIRMAN THOMAS: MOVED AND SECONDED. I

1 DON'T THINK WE NEED TO TAKE A VOTE ON THAT. SO
2 THANK YOU, EVERYBODY, FOR YOUR PARTICIPATION AS
3 ALWAYS. WE STAND ADJOURNED.

4 (THE MEETING WAS THEN CONCLUDED AT 11:26
5 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 MAY 25, 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
133 HENNA COURT
SANDPOINT, IDAHO
(208) 255-5453