

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

LOCATION: AS INDICATED ON THE AGENDA

DATE: AUGUST 20, 2015
9 A.M.

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

BRS FILE NO.: 97834

BARRISTERS' REPORTING SERVICE

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 PA 15-01 (LATE STAGE PRECLINICAL PROJECTS). | 5 |
| 4. PUBLIC COMMENT | NONE |
| 5. ADJOURNMENT | 14 |

BARRISTERS' REPORTING SERVICE

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AUGUST 20, 2015; 9 A.M.

CHAIRMAN THOMAS: I'D LIKE TO WELCOME EVERYBODY AND CALL THE MEETING OF THE GOVERNING BOARD AND THE APPLICATION REVIEW SUBCOMMITTEE TO ORDER. MOST OF US ARE ON VIA TELEPHONE. I KNOW JEFF IS ON-SITE THERE AT LEAST, IF THERE'S NOT OTHER MEMBERS, IN THE CIRM OFFICES, AND WE WELCOME EVERYBODY TO THIS DISCUSSION. MARIA, PLEASE CALL THE ROLL.

MS. BONNEVILLE: DAVID BRENNER. SUE BRYANT. KEN BURTIS. ANNE-MARIE DULIEGE.

DR. DULIEGE: HERE.

MS. BONNEVILLE: MICHAEL FRIEDMAN.

DR. FRIEDMAN: HERE.

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

DR. HIGGINS: HERE.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: HERE.

MS. BONNEVILLE: SHERRY LANSING. KATHY LAPORTE. BERT LUBIN. SHLOMO MELMED.

DR. MELMED: HERE.

MS. BONNEVILLE: LAUREN MILLER.

MS. MILLER: HERE.

BARRISTERS' REPORTING SERVICE

1 MS. BONNEVILLE: LLOYD MINER. ADRIANA
2 PADILLA.
3 DR. PADILLA: HERE.
4 MS. BONNEVILLE: JOE PANETTA.
5 MR. PANETTA: HERE.
6 MS. BONNEVILLE: ROBERT PRICE. FRANCISCO
7 PRIETO.
8 DR. PRIETO: HERE.
9 MS. BONNEVILLE: CARMEN PULIAFITO. ROBERT
10 QUINT.
11 DR. QUINT: PRESENT.
12 MS. BONNEVILLE: AL ROWLETT.
13 MR. ROWLETT: HERE.
14 MS. BONNEVILLE: JEFF SHEEHY.
15 MR. SHEEHY: HERE.
16 MS. BONNEVILLE: OS STEWARD.
17 DR. STEWARD: HERE.
18 MS. BONNEVILLE: JONATHAN THOMAS.
19 CHAIRMAN THOMAS: HERE.
20 MS. BONNEVILLE: ART TORRES. KRISTINA
21 VUORI. DONNA WESTON. DIANE WINOKUR.
22 MS. WINOKUR: HERE.
23 MS. BONNEVILLE: THANK YOU.
24 CHAIRMAN THOMAS: THANK YOU, MARIA. SO
25 WE HAVE ONE ITEM ON TODAY'S AGENDA, WHICH IS

BARRISTERS' REPORTING SERVICE

1 CONSIDERATION OF APPLICATIONS SUBMITTED IN RESPONSE
2 TO PA 15-01 WHICH ARE LATE STAGE PRECLINICAL
3 PROJECTS. WE'RE GOING TO NOW GO STRAIGHT TO THE
4 APPLICATIONS REVIEW SUBCOMMITTEE, WHICH IS CHAIRED
5 BY JEFF SHEEHY. SO, JEFF, IF I COULD TURN IT OVER
6 TO YOU AT THIS POINT.

7 CHAIRMAN SHEEHY: THANK YOU, J.T. ONE
8 QUICK HOUSEKEEPING QUESTION. ARE THERE MEMBERS OF
9 THE PUBLIC AT ANY OF THE SITES? OKAY.

10 SO WE'LL START WITH CONSIDERATION OF
11 PA 15-01, APPLICATION 08309. AND, DR. SAMBRANO, THE
12 WAY WE'LL DO IT IS DR. SAMBRANO WILL GIVE US A
13 DESCRIPTION OF THE PROJECT, AND THEN WE'LL TAKE
14 QUESTIONS, AND THEN WE'LL TAKE MOTIONS ON WHETHER TO
15 ACCEPT THE RECOMMENDATIONS.

16 DR. SAMBRANO: THANK YOU, MR. CHAIRMAN.
17 SO AS IT WAS STATED, WE ARE BRINGING FOR YOUR
18 CONSIDERATION RECOMMENDATIONS FROM THE GRANTS
19 WORKING GROUP ON TWO APPLICATIONS WHICH HAPPEN TO BE
20 RESPONDING TO PA 15-01 WHICH SUPPORTS PRECLINICAL
21 DEVELOPMENT ACTIVITIES LEADING TO INITIATION OF A
22 CLINICAL TRIAL. I JUST WANT TO REMIND ALL OF YOU OF
23 THE SCORING SYSTEM SO YOU HAVE SOME CONTEXT OF WHAT
24 THE SCORES WILL MEAN.

25 THE SCORING THAT IS USED BY THE GRANTS

BARRISTERS' REPORTING SERVICE

1 WORKING GROUP FOR A PRECLINICAL PROGRAM IS EITHER A
2 ONE, A TWO, OR A THREE. ONE MEANS THAT THE
3 APPLICATION IS OF EXCEPTIONAL MERIT OR IT WARRANTS
4 FUNDING. SCORE OF TWO MEANS IT NEEDS IMPROVEMENT,
5 DOESN'T WARRANT FUNDING AT THIS TIME, BUT COULD BE
6 RESUBMITTED BY THE APPLICANT IF THEY ADDRESS THOSE
7 AREAS OF CONCERN. AND A SCORE OF THREE MEANS THAT
8 THE APPLICATION IS SUFFICIENTLY FLAWED THAT IT
9 SHOULD NOT WARRANT FUNDING AND IS NOT SOMETHING THAT
10 THE GRANTS WORKING GROUP WOULD WANT TO RECONSIDER.

11 SO FOR THAT FIRST APPLICATION, WHICH IS
12 THE LSP1-08309, THIS IS A PRECLINICAL DEVELOPMENT
13 STUDY OF A SMALL MOLECULE FOR OSTEOARTHRITIS. THE
14 THERAPY ITSELF WOULD BE A SMALL MOLECULE DRUG THAT
15 PROMOTES CARTILAGE RESIDENT MESENCHYMAL STEM CELLS
16 TO DIFFERENTIATE HOPEFULLY INTO CHONDROCYTES.

17 INDICATION IS PATIENTS WITH OSTEOARTHRITIS
18 AND CARTILAGE INJURY.

19 AND THE GOAL OF THIS STUDY IS TO COMPLETE
20 IND-ENABLING STUDIES THAT SUPPORT THE SAFETY AND
21 EFFICACY OF THE CANDIDATE FOR CLINICAL STUDY, AND TO
22 FILE AN IND APPLICATION WITH THE FDA.

23 THERE ARE THREE MAJOR AREAS OF PROPOSED
24 ACTIVITIES WHICH INCLUDE GLP AND PRECLINICAL SAFETY
25 STUDIES; NON-GLP STUDIES TO DETERMINE THE MAXIMUM

BARRISTERS' REPORTING SERVICE

1 TOLERATED DOSE OF THE SMALL MOLECULE; AND, OF
2 COURSE, TO PREPARE THE IND DOCUMENT AND FILE IT.

3 FUNDS REQUESTED FOR THIS PROJECT ARE \$1.67
4 MILLION.

5 AND THE ASSESSMENT OF THIS APPLICATION BY
6 THE GRANTS WORKING GROUP WAS A UNANIMOUS ONE,
7 MEANING THEY THOUGHT IT WAS OF EXCEPTIONAL MERIT AND
8 WARRANTS FUNDING.

9 FOLLOWING THE GRANTS WORKING GROUP REVIEW,
10 WE, THE CIRM TEAM, CONSIDERED THAT RECOMMENDATION
11 AND WHETHER THERE ARE ADDITIONAL RECOMMENDATIONS
12 THAT WE WOULD LIKE TO BRING FORWARD. ESSENTIALLY WE
13 RECOMMEND THE SAME THING, THAT THIS SHOULD BE
14 FUNDED, AND WE CONCUR WITH THE GRANTS WORKING GROUP
15 RECOMMENDATION FOR THE AMOUNT REQUESTED.

16 MR. SHEEHY.

17 CHAIRMAN SHEEHY: SO DO ANY MEMBERS HAVE
18 ANY QUESTIONS ABOUT THE PRESENTATION? OKAY. THEN
19 COULD I GET A MOTION EITHER TO ACCEPT OR REJECT THE
20 GRANTS WORKING GROUP RECOMMENDATION?

21 DR. DULIEGE: I'M MAKING THE MOTION TO
22 ACCEPT.

23 CHAIRMAN SHEEHY: SO DR. DULIEGE HAS MADE
24 THE MOTION. DO WE HAVE A SECOND?

25 MR. JUELGAARD: I SECOND THE MOTION.

BARRISTERS' REPORTING SERVICE

1 CHAIRMAN SHEEHY: WE HAVE THE MOTION. ANY
2 FURTHER DISCUSSION? WE HAVE NO PUBLIC, SO NO PUBLIC
3 COMMENT. WE NEED A ROLL CALL. SO MR. HARRISON WILL
4 CALL THE ROLL OR MS. BONNEVILLE. SORRY.

5 MS. BONNEVILLE: ANNE-MARIE DULIEGE.

6 DR. DULIEGE: AYE.

7 MS. BONNEVILLE: DAVID HIGGINS.

8 DR. HIGGINS: YES.

9 MS. BONNEVILLE: STEVE JUELSGAARD.

10 DR. JUELSGAARD: YES.

11 MS. BONNEVILLE: SHERRY LANSING. KATHY
12 LAPORTE. LAUREN MILLER.

13 MS. MILLER: YES.

14 MS. BONNEVILLE: ADRIANA PADILLA.

15 DR. PADILLA: YES.

16 MS. BONNEVILLE: 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.

23 MR. ROWLETT: YES.

24 MS. BONNEVILLE: JEFF SHEEHY.

25 CHAIRMAN SHEEHY: YES.

BARRISTERS' REPORTING SERVICE

1 MS. BONNEVILLE: OS STEWARD.

2 DR. STEWARD: YES.

3 MS. BONNEVILLE: JONATHAN THOMAS.

4 CHAIRMAN THOMAS: YES.

5 MS. BONNEVILLE: ART TORRES. DIANE
6 WINOKUR.

7 MS. WINOKUR: YES.

8 DR. MELMED: YOU NEVER CALLED MY NAME.

9 MS. BONNEVILLE: DR. MELMED, IT'S FOR THE
10 APPLICATION REVIEW SUBCOMMITTEE ARE THE VOTING
11 MEMBERS.

12 DR. MELMED: OKAY. GOTCHA.

13 MR. HARRISON: MOTION CARRIES.

14 CHAIRMAN SHEEHY: MOTION CARRIES. THANK
15 YOU.

16 DR. SAMBRANO, WOULD YOU TAKE US THROUGH
17 THE NEXT APPLICATION, LSP1-08292?

18 DR. SAMBRANO: THANK YOU, MR. SHEEHY. SO
19 THIS NEXT APPLICATION IS A PRECLINICAL DEVELOPMENT
20 STUDY OF ADIPOCYTE STEM CELLS FOR LIVER
21 REGENERATION.

22 THE THERAPY IS A CELL THERAPY THAT IS
23 DERIVED FROM ADIPOCYTES AND TRANSFORMED INTO
24 HEPATOCYTE-LIKE CELLS.

25 THE INDICATION THAT IS PROPOSED FOR THE

BARRISTERS' REPORTING SERVICE

1 FIRST CLINICAL TRIAL IS ACUTE ALCOHOL-INDUCED LIVER
2 FAILURE WITHOUT PREEXISTING CIRRHOSIS.

3 THE GOAL OF THE STUDY IS TO COMPLETE
4 IND-ENABLING WORK SUPPORTING THE SAFETY AND EFFICACY
5 OF THIS CANDIDATE FOR CLINICAL STUDY AND TO FILE AN
6 IND WITH THE FDA.

7 THE MAJOR PROPOSED ACTIVITIES INCLUDE
8 DEVELOPMENT OF A METHOD TO MONITOR THE FUNCTION OF
9 THE TRANSPLANTED HEPATOCYTES IN PATIENTS, TO
10 DEMONSTRATE EFFICACY AND SAFETY OF THE INDUCED
11 HEPATOCYTES FOR LIVER REGENERATION IN A PRECLINICAL
12 MODEL, AND TO DEVELOP A METHOD FOR THE PRODUCTION OF
13 THESE ADIPOCYTE-DERIVED HEPATOCYTE-LIKE CELLS.

14 FUNDS REQUESTED ARE \$2.99 MILLION FOR THIS
15 PROPOSAL.

16 THE GRANTS WORKING GROUP UNANIMOUSLY GAVE
17 THIS A SCORE OF THREE, WHICH MEANS IT IS
18 SUFFICIENTLY FLAWED AND DOES NOT WARRANT FUNDING AT
19 THIS TIME. THE TEAM RECOMMENDATION IS ALSO DO NOT
20 FUND AND DO NOT ALLOW REAPPLICATION. THAT IS, WE
21 CONCUR WITH THE GRANTS WORKING GROUP.

22 CHAIRMAN SHEEHY: SO IS THERE ANY
23 DISCUSSION, ANY QUESTIONS FROM MEMBERS ABOUT THE
24 PRESENTATION? SO DO I HAVE A MOTION TO ADOPT OR
25 REJECT THE GWG RECOMMENDATION?

BARRISTERS' REPORTING SERVICE

1 DR. PRIETO: I'LL SO MOVE.

2 MR. SHEEHY: SECOND?

3 DR. JUELGAARD: I'LL SECOND THE MOTION.

4 DR. SHEEHY: IF THERE'S NO FURTHER
5 DISCUSSION, NO PUBLIC COMMENT, THEN WE CAN CALL THE
6 ROLL.

7 MR. HARRISON: JUST TO CLARIFY THAT THE
8 MOTION IS TO ACCEPT THE RECOMMENDATION NOT TO FUND
9 APPLICATION LSP1-08292 AND NOT TO ALLOW RESUBMISSION
10 OF THE SAME PROJECT.

11 CHAIRMAN SHEEHY: MS. BONNEVILLE.

12 MS. BONNEVILLE: ANNE-MARIE DULIEGE.

13 DR. DULIEGE: AYE.

14 MS. BONNEVILLE: DAVID HIGGINS.

15 DR. HIGGINS: YES.

16 MS. BONNEVILLE: STEVE JUELGAARD.

17 DR. JUELGAARD: YES.

18 MS. BONNEVILLE: SHERRY LANSING. KATHY
19 LAPORTE. LAUREN MILLER.

20 MS. MILLER: YES.

21 MS. BONNEVILLE: ADRIANA PADILLA.

22 DR. PADILLA: YES.

23 MS. BONNEVILLE: JOE PANETTA.

24 MR. PANETTA: YES.

25 MS. BONNEVILLE: FRANCISCO PRIETO.

BARRISTERS' REPORTING SERVICE

1 DR. PRIETO: AYE.
2 MS. BONNEVILLE: ROBERT QUINT.
3 DR. QUINT: YES.
4 MS. BONNEVILLE: AL ROWLETT.
5 MR. ROWLETT: YES.
6 MS. BONNEVILLE: JEFF SHEEHY.
7 MR. SHEEHY: YES.
8 MS. BONNEVILLE: OS STEWARD.
9 DR. STEWARD: YES.
10 MS. BONNEVILLE: JONATHAN THOMAS.
11 CHAIRMAN THOMAS: YES.
12 MS. BONNEVILLE: ART TORRES.
13 MR. TORRES: AYE.
14 MS. BONNEVILLE: DIANE WINOKUR.
15 MS. WINOKUR: YES.
16 MR. HARRISON: MOTION CARRIES 14 VOTES TO
17 ZERO.
18 MR. SHEEHY: SO, CHAIRMAN THOMAS, THAT
19 CONCLUDES THE BUSINESS OF THE APPLICATION REVIEW
20 SUBCOMMITTEE.
21 CHAIRMAN THOMAS: THANK YOU VERY MUCH,
22 MR. SHEEHY.
23 MAKE NOTE IT DOESN'T SOUND LIKE WE HAVE
24 ANY MEMBERS OF THE PUBLIC AT ANY OF THE SITES, BUT
25 JUST ONE LAST SHOT FOR GENERAL PUBLIC COMMENT ON ANY

BARRISTERS' REPORTING SERVICE

1 TOPIC. DO WE HAVE ANYBODY WHO WOULD LIKE TO SPEAK
2 AT THIS POINT?

3 MR. SHEEHY: WE DO HAVE SOMEONE IN SAN
4 FRANCISCO.

5 DR. MILLS: I'M NOT A MEMBER OF THE
6 PUBLIC. I JUST WANTED TO JUST MAKE A FEW QUICK
7 COMMENTS ABOUT THIS MEETING AND WHY THIS MEETING IS
8 SPECIAL TODAY. THIS IS THE FIRST OFF-BOARD MEETING
9 APPLICATION REVIEW SUBCOMMITTEE MEETING THAT WE'VE
10 HAD. DURING THE OTHER REVIEW CYCLES, IT ENDED UP
11 NOT BEING NECESSARY TO HAVE ONE OF THESE.

12 I JUST WANT TO LET EVERYONE KNOW HOW
13 APPRECIATIVE I AM FOR THEIR PARTICIPATION IN THIS
14 MEETING TODAY. OBVIOUSLY OUR MISSION IS TO
15 ACCELERATE STEM CELL TREATMENTS TO PATIENTS WITH
16 UNMET MEDICAL NEEDS. AS A RESULT, THAT HAS A TIME
17 COMPONENT TO IT. BY HAVING THIS MEETING TODAY AS A
18 CIRM COMMITTEE MEETING, WE HAVE REMOVED A FULL MONTH
19 OUT OF THE REVIEW AND APPROVAL CYCLE. AND IN A
20 SMALL PART, BUT IN A MEANINGFUL WAY, FULFILLING OUR
21 MISSION OF ACCELERATING THESE STEM CELL TREATMENTS.
22 SO WE'RE SENDING A CLEAR MESSAGE THAT SAYS TIME
23 MATTERS TO US. AND YOUR PARTICIPATION TODAY HELPED
24 US DO THAT. SO I JUST WANTED TO SAY THANK YOU FOR
25 DOING THAT.

BARRISTERS' REPORTING SERVICE

1 CHAIRMAN THOMAS: THANK YOU VERY MUCH, DR.
2 MILLS. AND CONTINUED THANKS FROM THE BOARD FOR THE
3 OUTSTANDING WORK THAT YOU AND YOUR TEAM HAVE DONE IN
4 DEVELOPING CIRM 2.0 THAT IS THE PROTOCOL THAT ALLOWS
5 FOR THIS EXPEDITED, ACCELERATED TREATMENT.

6 ANY OTHER COMMENTS BY MEMBERS OF THE
7 PUBLIC OR ANY COMMENTS? HEARING NONE, I THINK,
8 MEMBERS OF THE BOARD AND SUBCOMMITTEE, WE HAVE
9 CONCLUDED TODAY'S AGENDA. AND I WILL ENTERTAIN A
10 MOTION TO ADJOURN.

11 (MULTIPLE MOTIONS.)

12 CHAIRMAN THOMAS: MOVED AND SECONDED.
13 THANK YOU VERY MUCH. WE'LL SEE YOU IN SEPTEMBER.

14 (THE MEETING WAS THEN CONCLUDED AT
15 09:17 A.M.)

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BARRISTERS' REPORTING SERVICE

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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 OF THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE AND THE APPLICATION REVIEW SUBCOMMITTEE IN THE MATTER OF ITS REGULAR MEETING HELD ON AUGUST 20, 2015, 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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