

BETH C. DRAIN, CA CSR NO. 7152

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

LOCATION: VIA ZOOM

DATE: NOVEMBER 23, 2021
10 A.M.

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

FILE NO.: 2021-23

**133 HENNA COURT, SANDPOINT, IDAHO 83864
208-920-3543 DRAIBE@HOTMAIL.COM**

BETH C. DRAIN, CA CSR NO. 7152

I N D E X

ITEM DESCRIPTION	PAGE NO.
OPEN SESSION:	
1. CALL TO ORDER	3
2. ROLL CALL	3
ACTION ITEMS:	
3. CONSIDERATION OF APPLICATIONS SUBMITTED IN RESPONSE TO TRANSLATIONAL PROJECTS PROGRAM ANNOUNCEMENT (TRAN 1,2,3 OR 4):	4
4. CONSIDERATION OF APPLICATIONS SUBMITTED IN RESPONSE TO CLINICAL TRIAL STAGE PROJECTS PROGRAM ANNOUNCEMENT (CLIN 1,2 OR 3):	43
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 3 AND 4 ABOVE. (HEALTH & SAFETY CODE 125290.30(F) (3) (B) AND (C)).	
DISCUSSION ITEMS:	
6. PUBLIC COMMENT	NONE
7. ADJOURNMENT	52

BETH C. DRAIN, CA CSR NO. 7152

1
2
3
4
5
6
7
8
9
10
11
12
13
14
15
16
17
18
19
20
21
22
23
24
25

NOVEMBER 23, 2021; 10 A.M.

CHAIRMAN THOMAS: OKAY. THANK YOU. GOOD MORNING, EVERYBODY, TO THE NOVEMBER MEETING OF THE ICOC AND APPLICATION REVIEW SUBCOMMITTEE. MARIA, WILL YOU PLEASE CALL THE ROLL.

MS. BONNEVILLE: DAN BERNAL. ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: YSABEL DURON.

MS. DURON: PRESENT.

MS. BONNEVILLE: MARK FISCHER-COLBRIE.

DR. FISCHER-COLBRIE: HERE.

MS. BONNEVILLE: FRED FISHER. LEONDR CLARK-HARVEY. DAVID HIGGINS.

DR. HIGGINS: HERE.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: PRESENT.

MS. BONNEVILLE: RICH LAJARA.

MR. LAJARA: HERE.

MS. BONNEVILLE: DAVE MARTIN.

DR. MARTIN: HERE.

MS. BONNEVILLE: CHRISTINE MIASKOWSKI. LAUREN MILLER-ROGEN. ADRIANA PADILLA.

DR. PADILLA: HERE.

BETH C. DRAIN, CA CSR NO. 7152

1 MS. BONNEVILLE: JOE PANETTA.
2 MR. PANETTA: HERE.
3 MS. BONNEVILLE: AL ROWLETT.
4 MR. ROWLETT: PRESENT.
5 MS. BONNEVILLE: JONATHAN THOMAS.
6 CHAIRMAN THOMAS: HERE.
7 MS. BONNEVILLE: ART TORRES.
8 MR. TORRES: HERE.
9 MS. BONNEVILLE: KAROL WATSON.
10 I'D LIKE TO GO BACK TO FRED FISHER.
11 DR. FISHER: FRED IS HERE. SORRY ABOUT
12 THAT.
13 MR. MELMED: MARIA, YOU NEVER CALLED MY
14 NAME.
15 MS. BONNEVILLE: DR. MELMED. AND LARRY
16 GOLDSTEIN.
17 DR. GOLDSTEIN: YEAH. I AM HERE.
18 MS. BONNEVILLE: WE HAVE A QUORUM.
19 CHAIRMAN THOMAS: THANK YOU. PROCEED TO
20 ACTION ITEM NO. 3, CONSIDERATION OF APPLICATIONS
21 SUBMITTED IN RESPONSE TO TRANSLATIONAL PROJECTS
22 PROGRAM ANNOUNCEMENT TRANS 1, 2, 3, OR 4. AS
23 ALWAYS, WE HAVE A PRESENTATION FROM DR. SAMBRANO.
24 DR. SAMBRANO: THANK YOU, MR. CHAIRMAN.
25 GOOD MORNING TO ALL. I'M GOING TO SHARE MY SCREEN

BETH C. DRAIN, CA CSR NO. 7152

1 AND HOPE THAT THAT WILL COME UP IN JUST A SECOND.
2 OKAY. I'M GOING TO SHARE THIS NOT IN PRESENTATION
3 MODE BECAUSE I'VE HAD SOME ISSUES WITH THAT. SO
4 HOPEFULLY YOU CAN ALL SEE THIS.

5 SO THESE ARE THE GRANTS WORKING GROUP
6 RECOMMENDATIONS RELATED TO THE TRANSLATIONAL REVIEW
7 THAT OCCURRED LAST MONTH. AND SO JUST AS A
8 REMINDER, THE TRANSLATIONAL PROGRAM IS PART OF OUR
9 RECURRING CORE FUNDING OPPORTUNITIES FOR THERAPY
10 DEVELOPMENT THAT STEM FROM DISCOVERY STAGE WHERE WE
11 TAKE NEW IDEAS AND HAVE THE OPPORTUNITY TO SUPPORT
12 PROJECTS ALL THE WAY THROUGH THE CLINIC.

13 SPECIFICALLY THE TRANSLATIONAL PROGRAM
14 SUPPORTS STEM CELL-BASED PROJECTS OR GENE THERAPY
15 PROJECTS THAT ARE GOING TO ACCELERATE COMPLETION OF
16 THE TRANSLATIONAL STAGE ACTIVITIES THAT ARE NEEDED
17 FOR ADVANCEMENT TO CLINICAL STUDY. AND SO THE
18 PROGRAM SUPPORTS THE FOUR DIFFERENT PRODUCT TYPES.
19 SO IT WILL SUPPORT THERAPEUTICS, DIAGNOSTICS,
20 DEVICES, OR TOOLS. AND BECAUSE THE TRANSLATIONAL
21 ACTIVITIES FOR EACH OF THESE PRODUCT TYPES DIFFERS
22 AND THE TIMING AND COST CAN BE DIFFERENT, WE HAVE
23 TAILORED THE PROGRAM AROUND THOSE ELEMENTS.

24 SO THERAPEUTIC ALLOWS FOR THE MOST TIME,
25 UP TO 30 MONTHS, AND ALSO THE GREATEST AMOUNT OF

BETH C. DRAIN, CA CSR NO. 7152

1 FUNDING, PARTICULARLY FOR CELL THERAPIES OR
2 BIOLOGICS, AND UP TO 4 MILLION; WHEREAS, TOOLS ALLOW
3 ONLY UP TO 1 MILLION.

4 THE TRANSLATIONAL PROGRAM TAKES PROJECTS
5 THAT ARE AT A SINGLE CANDIDATE STAGE WHERE IT IS
6 THAT THEY HAVE IDENTIFIED A SINGLE PRODUCT
7 CANDIDATE. SO FOR A THERAPEUTIC IT MEANS THAT THEY
8 HAVE A CANDIDATE WITH DISEASE MODIFYING ACTIVITY
9 THAT'S READY FOR TRANSLATIONAL STUDIES. FOR A TOOL
10 OR DEVICE OR DIAGNOSTIC, THAT THEY HAVE SOME TYPE OF
11 PROTOTYPE WITH DEMONSTRATION OF PROOF OF CONCEPT.
12 THE TRANSLATIONAL AWARD WILL TAKE THEM THROUGH THOSE
13 KEY STUDIES THAT AT THE END OF THE AWARD WILL LEAD
14 THEM TO A PRE-IND MEETING IF IT'S A THERAPEUTIC OR
15 TO A PRESUBMISSION MEETING WITH THE FDA. IF IT'S A
16 DIAGNOSTIC OR DEVICE OR IF IT'S A TOOL, THEY ARE
17 READY FOR DESIGN TRANSFER TO MANUFACTURING SO THAT
18 THEY CAN COMMERCIALIZE THAT.

19 THIS IS JUST ANOTHER VIEW OF LARGELY THE
20 SAME THING LOOKING AT NOW THE TRAN PROGRAM AND
21 SPECIFICALLY THE TRAN1 THAT'S FOCUSED ON
22 THERAPEUTICS AND HOW IT IS BOOKENDED WITH DISCOVERY
23 AT ONE END AND CLIN1 AT THE OTHER. SO WE ARE TAKING
24 PROJECTS THAT, FOLLOWING THEIR IDENTIFICATION OF A
25 SINGLE CANDIDATE, WILL GET THEM TO A PRE-IND MEETING

BETH C. DRAIN, CA CSR NO. 7152

1 OVER THE COURSE OF 30 MONTHS. AND THEN ONCE THEY
2 DO, THAT WILL ALLOW THEM TO APPLY FOR A CLIN1 AWARD
3 TO TAKE THEM THROUGH IND-ENABLING ACTIVITIES TO FILE
4 AN IND. AND WE MOSTLY FOCUS ON THE TRAN1 BECAUSE
5 MOST OF THE APPLICATIONS THAT WE HAVE ARE TRAN1 THAT
6 ARE DEVELOPING A THERAPEUTIC. IN THIS PARTICULAR
7 COHORT THAT YOU'RE GOING TO SEE, THEY ARE VIRTUALLY
8 ALL TRAN1. THERE IS A SINGLE TRAN4 OR TOOL
9 APPLICATION AND NONE OF THE OTHER VARIETIES.

10 SO A REMINDER OF THE REVIEW CRITERIA THAT
11 THE GRANTS WORKING GROUP USES TO ASSIGN MERIT TO
12 THESE APPLICATIONS. THEY ASK THE FOLLOWING FIVE
13 QUESTIONS: DOES THE PROJECT HOLD THE NECESSARY
14 SIGNIFICANCE AND POTENTIAL FOR IMPACT? DO THEY HAVE
15 A SOUND RATIONALE? IS THE PROJECT WELL-PLANNED AND
16 DESIGNED? IS IT FEASIBLE? AND, FINALLY, DOES THE
17 PROJECT ADDRESS THE NEEDS OF THE UNDERSERVED? AND I
18 WILL HIGHLIGHT THAT LAST CRITERION SINCE IT'S STILL
19 RELATIVELY NEW, SOMETHING THAT HAS BEEN INCORPORATED
20 IN ALL OF OUR FUNDING OPPORTUNITIES OVER THE LAST
21 YEAR.

22 AND IN PARTICULAR, WE ASK THE GRANTS
23 WORKING GROUP TO ASSESS WHETHER THE PROJECT PLAN AND
24 DESIGN ADEQUATELY ADDRESSES AND ACCOUNTS FOR THE
25 INFLUENCE OF RACE, ETHNICITY, SEX, AND GENDER

BETH C. DRAIN, CA CSR NO. 7152

1 DIVERSITY, AND WOULD THE PROJECT OUTCOMES INFORM THE
2 DEVELOPMENT OF A PRODUCT OR A TOOL THAT SERVES THE
3 UNMET MEDICAL NEEDS OF A DIVERSE CALIFORNIA
4 POPULATION, INCLUDING UNDERSERVED RACIAL/ETHNIC
5 COMMUNITIES.

6 THE SCORING SYSTEM THAT IS USED TO ASSIGN
7 MERIT BASED ON THOSE CRITERIA IS A SCORE ON A SCALE
8 OF ONE TO A HUNDRED. A SCORE BETWEEN 85 AND A
9 HUNDRED, INCLUDING 85, MEANS THAT THE APPLICATION IS
10 RECOMMENDED FOR FUNDING IF FUNDS ARE AVAILABLE.
11 SCORES BELOW 85 MEAN THAT THE APPLICATION IS NOT
12 RECOMMENDED FOR FUNDING. SO ALL APPLICATIONS ARE
13 SCORED BY THE SCIENTIFIC MEMBERS OF THE GRANTS
14 WORKING GROUP, AND WE TAKE THE MEDIAN OF ALL THOSE
15 INDIVIDUAL GWG SCORES TO DETERMINE THE FINAL SCORE
16 FOR EACH APPLICATION.

17 NOW, FOR THIS COHORT OF APPLICATIONS THAT
18 WERE RECEIVED, HERE ARE THE GRANTS WORKING GROUP
19 RECOMMENDATIONS IN SUMMARY FORMAT. SO WE HAD 19
20 APPLICATIONS THAT WERE REVIEWED. THERE WERE TEN
21 THAT WERE SCORED 85 OR ABOVE AND, THUS, WERE
22 RECOMMENDED FOR FUNDING. THE TOTAL APPLICANT
23 REQUEST OF THOSE TEN APPLICATIONS TOTALS TO JUST
24 ABOVE 40 MILLION, AND THE FUNDS AVAILABLE ARE 52
25 MILLION BASED ON THE ANNUAL ALLOCATION FOR THIS

BETH C. DRAIN, CA CSR NO. 7152

1 PROGRAM.

2 OKAY. I WANT TO TELL YOU A LITTLE BIT
3 ABOUT THE MINORITY REPORTS. SO WE HAVE ONE
4 APPLICATION THAT HAS A MINORITY REPORT. JUST WANT
5 TO REMIND YOU WHAT THAT IS. UNDER PROP 14 ANY
6 APPLICATION THAT IS NOT RECOMMENDED FOR FUNDING BY
7 THE GWG, BUT WHICH HAD 35 PERCENT OR MORE OF THE
8 SCIENTIFIC MEMBERS SCORE TO FUND THE APPLICATION,
9 MUST INCLUDE A MINORITY REPORT. SO WHAT THE
10 MINORITY REPORT IS, IT IS A SUMMARY OF THE OPINION
11 OF THE REVIEWERS THAT SCORED THE APPLICATION 85 OR
12 ABOVE. AND WE INCLUDE THAT WITHIN THE BROADER
13 REVIEW SUMMARY. AND SO WE PUT THAT TOGETHER.

14 AND SO I'M GOING TO JUST POINT TO THE ONE
15 THAT HAS IT, WHICH IS APPLICATION 12907. SO THAT
16 ONE RECEIVED A SCORE OF 83; BUT AS YOU CAN SEE,
17 THERE WERE SIX MEMBERS WHO SCORED IT 85 OR ABOVE AND
18 SEVEN WHO DID NOT.

19 AND SO THIS PARTICULAR APPLICATION IS
20 ENTITLED "INVESTIGATIONAL NEW DRUG ENABLING STUDIES
21 OF THE WEARABLE THERAPEUTIC DEVICE FOR CARDIAC
22 REGENERATION AFTER MYOCARDIAL INFARCTION." THIS IS
23 A BIOLOGIC AND DEVICE COMBINATION INTENDED TO TREAT
24 PATIENTS AFTER AN MI, AND IT IS ONE THAT IS EXPECTED
25 TO STIMULATE CARDIAC TISSUE PROGENITOR CELLS IN

BETH C. DRAIN, CA CSR NO. 7152

1 ORDER TO REDUCE SCAR FORMATION AND RESTORE FUNCTION.
2 SO THAT IS THE APPLICATION.

3 LET ME JUST BRIEFLY READ TO YOU THE
4 MINORITY REPORT AS FOUND IN THE REVIEW SUMMARY,
5 WHICH STATES THAT REVIEWERS WHO SCORED THIS
6 APPLICATION 85 OR ABOVE NOTED THE INCREASING BURDEN
7 OF POST MYOCARDIAL INFARCTION, HEART FAILURE, AND
8 THE NOVELTY OF A REGENERATIVE RATHER THAN
9 AMELIORATIVE THERAPEUTIC APPROACH. THESE REVIEWERS
10 WERE ALSO OPTIMISTIC ABOUT THE DELIVERY OF A
11 SUBCUTANEOUS INJECTION, NOTING THAT THIS APPROACH
12 MAY BE PRACTICAL, WIDELY ADOPTABLE, PAID FOR BY
13 MEDICARE, AND REQUIRE NO ADDITIONAL PHYSICIAN
14 TRAINING.

15 INDIVIDUAL REVIEWERS WHO SCORED 85 OR
16 ABOVE WERE ALSO PLEASED WITH THE PRELIMINARY STUDIES
17 QUALIFICATION FOR THE PRINCIPAL INVESTIGATOR,
18 PARTNERSHIPS, APPROPRIATE GRADE MATERIAL, AND/OR
19 CONDUCT OF AN INTERACT MEETING WITH THE FDA. EXTANT
20 CONCERNS IN THIS GROUP INCLUDE OFF-TARGET EFFECTS OF
21 THE BIOLOGIC AND/OR POTENTIAL TIME DELAYS IN
22 TOXICITY TESTING OR DEVELOPMENT OF THE INJECTION
23 DEVICE.

24 AND THE MAJORITY OF THE REVIEWERS, AS
25 NOTED THERE, THE SEVEN, I THINK HAD CONCERNS ABOUT

BETH C. DRAIN, CA CSR NO. 7152

1 THE DESIGN OF THE PRECLINICAL STUDY; THAT IS, THE
2 DOSING STUDIES WITH THE ANIMALS, WHICH WAS WHAT I
3 THINK REALLY ULTIMATELY SPLIT THE GROUP. THE
4 APPLICANT HAS SUBMITTED A LETTER THAT ACKNOWLEDGES A
5 LOT OF THESE CRITICISMS AND HAS, AT LEAST IN
6 PRINCIPLE, AGREED TO MODIFY THEIR STUDY TO ALIGN
7 THEM WITH THE RECOMMENDATIONS BY THE GWG. SO WANTED
8 TO POINT THAT OUT SO THAT YOU CAN AND MAY HAVE A
9 PROGRAMMATIC DISCUSSION ABOUT IT.

10 FROM CIRM'S PERSPECTIVE, WE HAVE NO
11 SPECIFIC CONCERNS SHOULD YOU CHOOSE TO FUND THAT;
12 BUT THAT, OF COURSE, IS ULTIMATELY UP TO YOU. JUST
13 LIKE WITH OTHER APPLICANTS, WE WOULD WORK WITH THE
14 APPLICANTS TO MAKE ADJUSTMENTS TO MODIFY THE STUDY
15 AND ALIGN THE BUDGET WITH THOSE REQUIREMENTS.

16 SO LET ME STOP SHARING THE SLIDE DECK, AND
17 I WANT TO JUST SHARE, THEN, WITH YOU THE LIST OF ALL
18 APPLICATIONS SO THAT YOU CAN USE THAT AS A BASIS FOR
19 YOUR DISCUSSION. HOPEFULLY YOU CAN SEE THAT. MR.
20 CHAIRMAN.

21 CHAIRMAN THOMAS: THANK YOU, DR. SAMBRANO.

22 BEFORE WE GET TO THE VOTING, I JUST HAVE A
23 GENERAL QUESTION FOR YOU. SO NOT INFREQUENTLY WHEN
24 SOMEBODY SENDS A LETTER IN IN REGARDS TO AN
25 APPLICATION THAT WAS NOT RECOMMENDED FOR FUNDING,

BETH C. DRAIN, CA CSR NO. 7152

1 THE LETTERS PURPORT TO ADDRESS THE CONCERNS OF THE
2 GWG IN A FASHION THAT HOPES TO GET THE BOARD TO SEE
3 THAT THE ISSUES THAT MADE IT NOT RECOMMENDED FOR
4 FUNDING HAVE BEEN ADDRESSED. HOW EASY OR HARD IS IT
5 FOR YOU AND YOUR TEAM, AS YOU READ THESE LETTERS, TO
6 FIGURE OUT IF, INDEED, WHAT THEY'RE SAYING
7 ADEQUATELY ADDRESSEES THE GWG CONCERNS?

8 DR. SAMBRANO: WELL, I THINK IT IS
9 CHALLENGING BECAUSE TYPICALLY APPLICANTS ARE
10 ADDRESSING CONCERNS OF THE GWG. AND SO CLEARLY WE
11 CAN'T SPEAK FOR THE SCIENTIFIC REVIEWERS AND DON'T
12 NECESSARILY HAVE THE NECESSARY EXPERTISE IN MANY
13 CASES TO MAKE A FINAL JUDGMENT AS TO WHETHER THE
14 COMMENTS ARE ADEQUATELY ADDRESSED. I THINK THE
15 LETTERS ALSO DON'T ALWAYS NECESSARILY CONTAIN DATA
16 TO BACK UP STATEMENTS. SO I THINK, IN GENERAL, WE
17 LOOK TO THOSE LETTERS AS BEING AT LEAST AN EFFORT OR
18 A GOOD-FAITH EFFORT TO TRY TO RECONCILE SOME OF THE
19 CONCERNS THAT WERE BROUGHT UP BY REVIEWERS. SO I
20 THINK TAKEN AT FACE VALUE IT'S GREAT.

21 IN GENERAL, WE LIKE TO SEE APPLICANTS
22 RESUBMIT, INCLUDE DATA AND THOROUGH COMMENT THAT THE
23 GWG CAN LOOK AT AGAIN IN ORDER TO MAKE AN ASSESSMENT
24 AS TO WHETHER IT DOES ADDRESS THE CONCERNS THAT THEY
25 HAD. I THINK SOME OF IT MAY DEPEND ON WHAT THE

BETH C. DRAIN, CA CSR NO. 7152

1 CONCERNS ARE. I THINK IN SOME CASES, SUCH AS WITH
2 THE ONE THAT I JUST PRESENTED THAT HAS THE MINORITY
3 REPORT, THE CONCERNS REALLY RELATE TO HOW THE DESIGN
4 OF THE STUDIES WERE DONE WHICH ARE LARGELY PRETTY
5 STRAIGHTFORWARD. I THINK THE LETTER, IN GENERAL,
6 SPEAKS TO AN INTEREST IN REDESIGNING THEM. IT
7 DOESN'T GIVE THE SPECIFICS AS TO EXACTLY WHAT IT
8 WILL BE; BUT, AS I MENTIONED, THAT'S SOMETHING THAT
9 WE CAN WORK WITH THE APPLICANT WITH. I THINK IN
10 OTHER CASES WHERE DATA IS REQUIRED, IT WILL BE MUCH
11 MORE DIFFICULT FOR US TO REALLY COMMENT OR HAVE AN
12 OPINION NECESSARILY.

13 CHAIRMAN THOMAS: OKAY. THANK YOU. SO,
14 MEMBERS OF THE BOARD AND THE APPLICATION REVIEW
15 SUBCOMMITTEE, WHAT WE'RE GOING TO DO HERE IS A
16 FOUR-PRONGED APPROACH. FIRST, I'M GOING TO ASK IF
17 THERE ARE ANY APPLICATIONS THAT ARE NOT RECOMMENDED
18 FOR FUNDING BE MOVED TO THE RECOMMENDED FOR FUNDING
19 CATEGORY. SECONDLY, I'M GOING TO ASK IF THERE ARE
20 ANY APPLICATIONS THAT ARE RECOMMENDED FOR FUNDING OR
21 RECOMMENDED TO BE REMOVED FROM THAT CATEGORY TO NOT
22 RECOMMENDED. THIRD, HAVING DONE ONE AND TWO, I WILL
23 ASK FOR A VOTE TO CONFIRM NOT RECOMMENDING THOSE
24 PROJECTS IN THE NOT RECOMMENDED FOR FUNDING
25 CATEGORY. AND, FINALLY, NO. 4 WILL BE A VOTE ON

BETH C. DRAIN, CA CSR NO. 7152

1 APPROVING THOSE THAT HAVE BEEN RECOMMENDED.

2 SO SORRY FOR THE SOMEWHAT CONVOLUTED
3 PROCESS; BUT, AS YOU KNOW, THIS IS WHAT WE HAVE TO
4 DO IN THESE VOTING MOMENTS HERE.

5 OKAY. SO FIRST, ARE THERE ANY OF THE
6 PROJECTS NOT RECOMMENDED FOR FUNDING THAT ANYBODY
7 WOULD LIKE TO MOVE UP TO THE RECOMMENDED FOR FUNDING
8 CATEGORY?

9 MS. BONNEVILLE: J.T., MARK HAS HIS HAND
10 RAISED.

11 CHAIRMAN THOMAS: THANK YOU, MARIA. MARK.

12 DR. FISCHER-COLBRIE: I'D LIKE TO GIVE
13 CONSIDERATION TO 12889 RE THERAPEUTICS WITHIN THE
14 CONTEXT THAT IN THEIR LETTER OF RESPONSE --

15 MS. BONNEVILLE: MARK --

16 CHAIRMAN THOMAS: MARK, YOU JUST NEED A
17 SECOND BEFORE YOU CONTINUE YOUR EXPLANATION.

18 DR. FISCHER-COLBRIE: MY APOLOGIES.

19 MS. BONNEVILLE: MARK --

20 MR. TORRES: WHAT'S THE MOTION?

21 MS. BONNEVILLE: THERE'S NOT A MOTION ON
22 THE TABLE. MARK, YOU CANNOT MAKE A MOTION ON THIS
23 APPLICATION AND YOU CANNOT COMMENT EITHER.

24 MR. TORRES: CAN I MAKE A MOTION ON THIS
25 APPLICATION?

BETH C. DRAIN, CA CSR NO. 7152

1 MS. BONNEVILLE: NO, ART, YOU CANNOT.

2 MR. TORRES: HOW AM I CONFLICTED OUT ON
3 THIS ONE?

4 MS. BONNEVILLE: AS I MENTIONED, IF YOU
5 HAVE A CONFLICT WITH ANY OF THEM, THE ONLY MOTION OR
6 THE ONLY THING THAT YOU CAN DO AT THE END IS VOTE
7 EITHER YES OR NO FOR ALL OF THOSE EXCEPT FOR THOSE
8 WITH WHICH YOU HAVE A CONFLICT.

9 MR. TORRES: I'M JUST CONFUSED. I DON'T
10 RECALL HAVING A CONFLICT; BUT IF I DO, I WOULD
11 CERTAINLY ADHERE TO IT. IF SOMEONE WOULD JUST SEND
12 ME A QUICK NOTE AS TO WHAT THE CONFLICT IS.

13 MS. BONNEVILLE: SURE.

14 MR. TORRES: THANK YOU.

15 CHAIRMAN THOMAS: FOR PURPOSES OF
16 DISCUSSION, I'LL MOVE THAT WE MOVE THAT PARTICULAR
17 APPLICATION UP TO THE RECOMMENDED FOR FUNDING
18 CATEGORY. DO WE HAVE A SECOND? OKAY. DO NOT HEAR
19 A SECOND. MARIA, DO YOU SEE ANY HANDS UP THERE?

20 MS. BONNEVILLE: I DO NOT SEE ANY HANDS
21 RAISED.

22 CHAIRMAN THOMAS: OKAY. ARE THERE ANY
23 OTHER APPLICATIONS THAT ANYBODY WOULD LIKE TO MOVE
24 UP TO THE RECOMMENDED FOR FUNDING CATEGORY?

25 MR. TORRES: AM I CONFLICTED ON 12907 AS

BETH C. DRAIN, CA CSR NO. 7152

1 WELL, MARIA?

2 MS. BONNEVILLE: ART, YES.

3 MR. TORRES: OKAY.

4 CHAIRMAN THOMAS: OKAY. HEARING NONE, I
5 WOULD JUST LIKE TO MAKE SURE THAT ALL OF THE
6 APPLICANTS WHOSE APPLICATIONS ARE IN THE NOT
7 RECOMMENDED FOR FUNDING CATEGORY THAT, CORRECT ME IF
8 I'M WRONG, DR. SAMBRANO, THE NEXT APPLICATION DATE
9 FOR THESE TRAN AWARDS IS DECEMBER 7TH; IS THAT
10 CORRECT?

11 DR. SAMBRANO: THAT'S CORRECT, YES.

12 CHAIRMAN THOMAS: OKAY. SO TO ALL OF YOU
13 I WOULD RECOMMEND THAT, AS ALWAYS, YOU TAKE A LOOK
14 AT THE GWG COMMENTS. AND SHOULD YOU DECIDE TO
15 REAPPLY, FACTOR THOSE INTO YOUR RESUBMISSIONS AND
16 APPLY BY DECEMBER 7TH. AND, GIL, THE DATE FOR THE
17 HEARING OF THE NEXT GWG AND THESE APPLICATIONS IS
18 MORE OR LESS WHAT DATE AT THIS POINT?

19 DR. SAMBRANO: FOR THE NEXT CYCLE THE
20 REVIEW WOULD BE SCHEDULED IN MARCH AND THE BOARD
21 CONSIDERATION IN APRIL.

22 CHAIRMAN THOMAS: OKAY. SO EVERYBODY
23 LISTENING, YOU HAVE THE DATES AND PLEASE PROCEED
24 ACCORDINGLY.

25 OKAY. THE SECOND PART OF THIS FOUR-PART

BETH C. DRAIN, CA CSR NO. 7152

1 PROCESS, AS I SAID, ARE THERE ANY PROJECTS
2 RECOMMENDED FOR FUNDING THAT YOU WANT TO MOVE OUT OF
3 THE RECOMMENDED FOR FUNDING CATEGORY?

4 OKAY. HEARING NONE, THIRD PRONG. DO I
5 HEAR A MOTION TO CLOSE OUT THE NOT RECOMMENDED FOR
6 FUNDING CATEGORY? I'LL MAKE THAT MOTION.

7 MR. ROWLETT: SO MOVED. THIS IS AL
8 ROWLETT. OR I SECOND J.T.'S MOTION.

9 CHAIRMAN THOMAS: THANK YOU, AL. IS THERE
10 ANY DISCUSSION BY MEMBERS OF THE BOARD?

11 MS. DURON: YES, J.T.

12 CHAIRMAN THOMAS: YES, YSABEL.

13 MS. DURON: THANK YOU. NOT BEING THE
14 SCIENTIST IN THE ROOM, I WANTED TO ASK PERHAPS A FEW
15 OTHERS TO MAKE COMMENTS. IN READING THINGS LIKE THE
16 12920, 12883, AND A COUPLE OF THOSE ABOVE IT, I
17 THINK OF THEM AS SEEMING TO BE VERY EXCITING NEW
18 THERAPY OR ADVANCED THERAPY OR NEW SCIENCE THAT IS
19 EXCITING. OR IS THIS JUST SOME CONTINUATION OF WORK
20 THAT HAS BEEN DONE? FOR INSTANCE, ON THE 12, I
21 THINK THAT'S A, 967, THE B-CELL ACUTE LYMPHOBLASTIC
22 LEUKEMIA, I THINK MYSELF FOR INTERPRETATION AND FOR
23 THE IMPACT ON UNDERSERVED COMMUNITIES, I KNOW, FOR
24 INSTANCE, THAT NEW RESEARCH IS SHOWING LATINO KIDS
25 AT HIGHER RISK. THERE'S A MARKER THEY HAVE FOR

BETH C. DRAIN, CA CSR NO. 7152

1 LEUKEMIA THAT PUTS THEM AT HIGHER RISK BOTH IN
2 INCIDENCE AND LACK OF RESPONSE TO TREATMENT AS WELL
3 AS MORTALITY.

4 SO THIS KIND OF EXCITES ME, THE IDEA THAT
5 THERE IS THIS KIND OF A THERAPY BECOMING AVAILABLE
6 OR BEING TESTED OR AT LEAST PILOTED.

7 SO I'M ASKING SOME OF MY COLLEAGUES HERE
8 TO TELL ME IF THEY THINK THAT THERE IS SOME, AMONGST
9 THESE, THERE ARE SOME REALLY EXCITING NEW STEPS
10 FORWARD THAT WILL EVENTUALLY HELP VARIOUS PATIENTS
11 WHO ARE IN NEED OF THIS KIND OF WORK, CARE,
12 TREATMENT, THERAPY.

13 CHAIRMAN THOMAS: GIL, DO YOU WANT TO
14 ANSWER THAT QUESTION?

15 DR. SAMBRANO: I'M HAPPY TO SAY THAT I
16 THINK FROM CIRM'S PERSPECTIVE WE ARE EXCITED ABOUT
17 THESE PROJECTS. AND SO MANY OF THEM DO REPRESENT
18 PROJECTS THAT EITHER WE HAVE FUNDED PREVIOUSLY AND
19 ARE CONTINUING IN THEIR ENDEAVOR TO DEVELOP NEW
20 THERAPIES SUCH AS THE ONE THAT YOU CITED FOR THE
21 B-CELL ALL. SO, YES, I THINK MANY OF THEM DO.
22 SOME OF THEM ARE ALSO FOR SOME RARE DISEASES, THAT
23 ALTHOUGH THEY MAY HAVE LIMITED NUMBER OF PATIENTS
24 THAT THEY MIGHT ACT ON INITIALLY, THEY DO ALSO
25 REPRESENT A GENERALIZED APPROACH OR AN APPROACH THAT

BETH C. DRAIN, CA CSR NO. 7152

1 CAN BE GENERALIZED TO OTHER SIMILAR DISEASES. SO I
2 THINK FROM CIRM'S PERSPECTIVE, WE ARE PLEASED AND
3 HAPPY TO GO FORWARD WITH THESE.

4 CHAIRMAN THOMAS: OKAY. THANK YOU.
5 LARRY, GOT YOUR HAND UP?

6 DR. GOLDSTEIN: I WONDER IF I MAY MAKE
7 SOME REMARKS ABOUT WHETHER AND HOW OFTEN WE SHOULD
8 BE OVERRIDING THE EXPERT REVIEWERS, WHICH I THINK
9 PARTLY ADDRESSES YSABEL'S COMMENTS. IS THAT OKAY
10 WITH YOU, J.T.?

11 CHAIRMAN THOMAS: CERTAINLY.

12 DR. GOLDSTEIN: SO I THINK ONE OF THE
13 ISSUES IS -- LET ME START THIS WAY. ALL OF THESE
14 PROJECTS HAVE REALLY IMPORTANT GOALS. ALL OF THEM
15 USE INTERESTING METHODOLOGIES, SOME VERY NOVEL.
16 WHAT WE GET BACK FROM THE REVIEWERS IS NOT JUST THE
17 LEVEL OF EXCITEMENT. WE ACTUALLY GET BACK COMMENTS
18 ON HOW FEASIBLE IS THE PROJECT RELATIVE TO THE PLAN
19 THAT WAS PRESENTED BECAUSE YOU CAN HAVE GREAT GOALS;
20 BUT IF YOU DON'T HAVE A GOOD MAP FOR GETTING THERE,
21 IT'S NOT GOING TO BE SUCCESSFUL.

22 THE OTHER ISSUES ARE WE DON'T HAVE THE
23 GRANTS IN FRONT OF US SO WE REALLY CAN'T JUDGE HOW
24 IMPORTANT ARE THE CONSIDERATIONS THE REVIEWERS
25 RAISED, BUT I THINK WE HAVE TO TRUST THE REVIEWERS

BETH C. DRAIN, CA CSR NO. 7152

1 ON THAT. AND I'D SAY THAT MOST OF US ON THIS BOARD
2 DON'T HAVE THE DOMAIN-SPECIFIC EXPERTISE TO JUDGE
3 HOW IMPORTANT THOSE FLAWS ARE AND ARE THEY
4 ADEQUATELY REBUTTED, SAY, IN A REBUTTAL LETTER.

5 THE OTHER COMMENT I WANT TO MAKE IS WE
6 SOMETIMES MAKE IT SOUND AS THOUGH IF WE DON'T FUND A
7 GRANT NOW, IT WILL NEVER BE FUNDED. AND THE FACT IS
8 MY LONG EXPERIENCE WITH GRANTING AGENCIES WAS YOU
9 SOMETIMES GET A REJECTION, YOU IMPROVE THE PROJECT,
10 YOU SEND IT IN FOR REVIEW, AND IT OFTEN GETS FUNDED
11 AT THAT SECOND CHANCE. IF YOU LOOK AT THE GRANTS
12 THAT ARE IN GREEN IN THIS ROUND, FULLY HALF OF THOSE
13 GRANTS, FIVE OUT OF TEN, ARE RESUBMISSIONS. SO
14 THOSE ARE GRANTS THAT DIDN'T LET THE REVIEWS STOP
15 THEM FOREVER. THEY CAME UP WITH A GOOD SET OF
16 ADDRESSING OF THE REVIEWS. THEY WERE EXPERTLY
17 REVIEWED BY THE GWG, AND, IN FACT, THEY MADE IT INTO
18 THE FINAL FUNDING BATCH.

19 SO WHEN WE SAY NO, IT'S ONLY A DELAY OF A
20 FEW MONTHS POTENTIALLY. AND I THINK THAT'S WORTH IT
21 TO GET PROJECT PLANS THAT REALLY ARE JUDGED FEASIBLE
22 AND LIKELY TO SUCCEED ON AN IMPORTANT GOAL. SO
23 THANK YOU.

24 CHAIRMAN THOMAS: I THINK, LARRY, THOSE
25 ARE ALL VERY IMPORTANT OBSERVATIONS. AND I WOULD

BETH C. DRAIN, CA CSR NO. 7152

1 JUST ADD THAT IT'S PART OF THE BEAUTY OF THE CIRM
2 PROCESS THAT'S IN PLACE IS IT ALLOWS FOR PRECISELY
3 WHAT LARRY IS SAYING, WHICH IS THE IMPROVEMENT OF
4 SUBMISSIONS, FACTORING IN THE EXPERT COMMENTS WE GET
5 FROM THE GWG. FOR THOSE OF YOU WHO NEVER SAT
6 THROUGH ONE OF THE GWG MEETINGS, THE ATTENTION TO
7 ALL DETAIL IS METICULOUS AND TREMENDOUS EXPERTISE IS
8 ALWAYS BROUGHT TO BEAR. SO THE BOARD IS GETTING THE
9 BENEFIT OF THE BEST ADVICE OF EXTREMELY QUALIFIED
10 PEOPLE WHO SPEND A GREAT DEAL OF TIME AND ENERGY IN
11 EVALUATING EVERYTHING THAT COMES BEFORE US AND IS
12 RECOMMENDED FOR FUNDING OR NOT. BUT FOR THOSE THAT
13 AREN'T, WE ARE ALL ABOUT TRYING TO MAXIMIZE THE
14 CHANCE THAT A PROJECT IS GOING TO WORK. SO TO THE
15 EXTENT THAT IT CAN BE IMPROVED, RESUBMITTED, AND
16 EVALUATED AND GLEANED TO BE SOMETHING WORTHY OF
17 FUNDING, THAT JUST MAKES THE PROJECT STRONGER AND
18 THE PROGRAM THAT CIRM HAS STRONGER. SO I TOTALLY
19 ECHO EVERY SINGLE THING THAT LARRY SAID. THOSE ARE
20 ALL VERY IMPORTANT POINTS.

21 MS. DURON: J.T.

22 CHAIRMAN THOMAS: YES, YSABEL.

23 MS. DURON: FIRST OF ALL, THANKS TO LARRY
24 FOR THE PRIMER. I WILL PROBABLY NEED IT EVERY SIX
25 MONTHS OR SO, JUST A CONTINUATION TO REMIND ME.

BETH C. DRAIN, CA CSR NO. 7152

1 SECOND OF ALL, I WOULD LIKE TO SAY THAT
2 FROM MY PERSPECTIVE AS A CHAIR OF THE COMMUNICATIONS
3 SUBCOMMITTEE, I WANT TO UNDERSTAND THESE IN A WAY
4 THAT I CAN TRANSLATE THEM TO THE PUBLIC. I WANT TO
5 UNDERSTAND THAT I CAN GO OUT AND REALLY CONVINCED
6 THEM THAT THE DOLLARS THAT THEY'RE INVESTING HAVE
7 THIS HOPE AND THIS GREAT EXCITEMENT ABOUT THEM. AND
8 SO I'M LOOKING FOR THAT TOO.

9 I THINK IT'S WELL AND GOOD THAT WE ARE
10 HELPING SUPPORT THE SCIENCE, BUT THE PUBLIC NEEDS TO
11 UNDERSTAND WHY THE SCIENCE IS GOOD FOR THEM. AND I
12 DON'T THINK WE, AS CIRM, AS HARD AS WE HAVE TRIED,
13 HAVE EMBEDDED THAT EXCITEMENT IN THIS WORK AND MADE
14 THE PUBLIC FEEL GOOD ABOUT WHAT WE ARE DOING. SO
15 THANK YOU FOR THAT. I'LL REMEMBER THAT. AND
16 HOPEFULLY WE CAN COME UP WITH SOME WONDERFUL
17 COMMUNICATIONS PLANS TO REALLY REFLECT SOME OF THIS.

18 I WOULD GO IMMEDIATELY BACK TO THE LATINO
19 COMMUNITY AND SAY WE'VE GOT THIS HIGH INCIDENCE OF
20 PEDIATRIC CANCER, BUT THERE ARE SOME THINGS ON THE
21 HORIZON THAT MIGHT REALLY MAKE A DIFFERENCE TO SAVE
22 YOUR CHILD. SO THAT'S THE KIND OF A RELATIONSHIP I
23 WANT TO HAVE BETWEEN THE SCIENCE AND WITH THE
24 COMMUNITIES THAT WE HAVE TO TALK TO. SO THANKS,
25 LARRY. I APPRECIATE IT. AND THANK YOU, J.T., FOR

BETH C. DRAIN, CA CSR NO. 7152

1 THE TIME.

2 CHAIRMAN THOMAS: AND VERY VALID POINTS,
3 YSABEL. THE COMMUNICATION ASPECT IS CRITICAL, AND
4 WE HAVE A GREAT STORY TO TELL. BY THE WAY, WE ARE
5 DOING AN EXCELLENT JOB ON MANY FRONTS. FOR EXAMPLE,
6 I WOULD REFER YOU TO KEVIN AND KEVIN'S BLOG, WHICH
7 COMES OUT WHICH IS EXTREMELY UNDERSTANDABLE, BREAKS
8 DOWN THE PROJECTS, GETS ACROSS THE LEVEL OF
9 EXCITEMENT. NOW OBVIOUSLY THE BLOG CAN'T DEAL WITH
10 EVERY SINGLE PROJECT WE'VE APPROVED; BUT AS A FORM
11 OF COMMUNICATION, IT'S EXTREMELY READABLE AND I
12 THINK DOES SUCCEED IN BOTH GETTING ACROSS THE VERY
13 EXCELLENT WORK THAT WE ARE ENABLING AND ALSO DOES
14 CONVEY A SUFFICIENT LEVEL OF ENTHUSIASM.

15 BUT TO YOUR POINT, YSABEL, WE ALWAYS CAN
16 IMPROVE, FOR SURE, AND NEED TO SO THAT THE PUBLIC
17 UNDERSTANDS BOTH WHAT'S AVAILABLE AND, SINCE
18 TAXPAYERS ARE FUNDING THIS, THEY DESERVE TO KNOW
19 WHAT'S GOING ON. SO ALL THE ABOVE IS TRUE. SO
20 THANK YOU VERY MUCH FOR YOUR COMMENTS.

21 OKAY. SO LET'S SEE. I THINK --

22 MS. BONNEVILLE: DAVE MARTIN HAD HIS HAND
23 RAISED. IT'S DOWN NOW, BUT I WANTED TO MAKE SURE
24 THAT HE HAD AN OPPORTUNITY IF HE HAD A QUESTION.
25 SORRY TO INTERRUPT YOU.

BETH C. DRAIN, CA CSR NO. 7152

1 CHAIRMAN THOMAS: SURE. DAVE.

2 DR. MARTIN: I JUST WANTED TO MAKE A
3 COMMENT. I CERTAINLY AGREE WITH EVERYTHING THAT DR.
4 GOLDSTEIN STATED. BUT ON THE OTHER HAND, THE TIME
5 OF THIS GROUP AS A SCIENTIFIC REVIEW AND BEING
6 BLINDED AS A MEMBER OF THIS COMMITTEE IN TERMS OF,
7 THE SUBCOMMITTEE, IN TERMS OF WHO ARE THE REVIEWERS
8 ON THE GWG AND WHAT IS THEIR VERY SPECIFIC EXPERTISE
9 RAISES IN MY MIND -- I'VE GOT ABOUT THREE PAGES OF
10 QUESTIONS ON THESE THAT MOSTLY PERTAIN TO VERY
11 SPECIFIC QUESTIONS ABOUT WHAT IS THE TARGET. IS
12 THIS A VALID TARGET? DOES THIS GROUP NEED TO REALLY
13 GO AFTER AN INTERACT MEETING BEFORE THEY FILE A
14 PRE-IND SUBMISSION OR FOR A MEETING?

15 AND SO I'M SORT OF TORN BECAUSE WHEN I
16 LOOK AT THESE, I ALWAYS AS A SCIENTIST HAVE
17 QUESTIONS THAT GO BEYOND JUST THEY'RE FALLING INTO A
18 CATEGORY ABOVE 84. I'M SORRY. IT'S A LITTLE
19 COMPLICATED FOR US TO, I THINK, NAVIGATE THIS AS A
20 SCIENTIFIC REVIEW COMMITTEE.

21 CHAIRMAN THOMAS: THANK YOU, DAVE.

22 OTHER GENERAL COMMENTS FURTHER TO THESE
23 THEMES FROM MEMBERS OF THE BOARD? OKAY. SO I
24 THINK, MARIA, IF I'M CORRECT, WE HAD A MOTION TO
25 CLOSE OUT THE NOT APPROVED FOR FUNDING?

BETH C. DRAIN, CA CSR NO. 7152

1 MS. BONNEVILLE: THAT'S CORRECT. WE STILL
2 NEED PUBLIC COMMENT BEFORE THE VOTE.

3 CHAIRMAN THOMAS: CORRECT. SO IS THERE
4 ANY DISCUSSION ON THAT MOTION BY MEMBERS OF THE
5 BOARD? ANY COMMENTS FROM MEMBERS OF THE PUBLIC?
6 MARIA, I SEE A HAND. WE HAVE ONE COMMENT. IS THAT
7 WHAT YOU'RE SEEING?

8 MS. BONNEVILLE: WE HAVE TWO NOW. WHY
9 DON'T WE START. AS A REMINDER, IT'S THREE MINUTES
10 FOR PUBLIC COMMENT.

11 CHAIRMAN THOMAS: CORRECT. OKAY. WE'RE
12 GOING TO GO WITH WHOMEVER IS ATTACHED TO
13 (415) 235-2753. PLEASE STATE YOUR NAME,
14 AFFILIATION, AND, AGAIN, YOU HAVE THREE MINUTES.

15 MR. MCCORMACK: THIS IS KEVIN MCCORMACK,
16 THE COMMUNICATIONS DIRECTOR. I'M READING A LETTER
17 FROM DON REED IN SUPPORT OF DR. TOM OKARMA. SO THIS
18 IS DON'S LETTER.

19 "WE ALL KNOW TOM OKARMA, A GIANT IN THE
20 FIELD OF STEM CELL RESEARCH. HE HAS A RECORD OF
21 SUCCESS GOING BACK DECADES. AFTER HIS PIONEERING
22 WORK WITH GERON, OKARMA FOUNDED ASTERIAS AND
23 CONTINUED THE ONLY REGENERATIVE THERAPY FOR SPINAL
24 CORD INJURY AS WELL AS A STEM CELL DEVICE, CANCER
25 IMMUNOTHERAPY ON CLINICAL DEVELOPMENT FOR LUNG

1 CANCER.

2 "WE ALSO ALL KNOW SOMEONE WHO SUFFERS FROM
3 A MYOCARDIAL INFARCTION, A HEART ATTACK. EVEN WITH
4 THE CURRENT STANDARD OF CARE, HEART ATTACKS ALL TOO
5 OFTEN DEVELOP INTO CHRONIC HEART FAILURE. THIS CAN
6 NOT ONLY BE FATAL, BUT IS STAGGERINGLY EXPENSIVE, A
7 CONDITION THAT WILL SOON REACH 70 BILLION A YEAR IN
8 AMERICA ALONE. THIS DISEASE AND ITS ACCOMPANYING
9 COSTS DISPROPORTIONATELY AFFLICT UNDERSERVED
10 COMMUNITIES.

11 "THE PROJECT BEING CONSIDERED INVOLVES A
12 BIOLOGICAL DRUG THAT STIMULATES HEART REGENERATION
13 AFTER A HEART ATTACK TO RESTORE THE USEFUL FUNCTION
14 OF A DAMAGED HEART. THAT IS A BATTLE FOR LIFE
15 ITSELF. A BIG PLUS FOR THE PROPOSAL IS THE EASE OF
16 ITS IMPLEMENTATION. IT DOES NOT REQUIRE SURGERY NOR
17 SPECIAL EQUIPMENT NOR PHYSICAL TRAINING. IT IS
18 ADMINISTERED THROUGH WHAT THEY CALL A WEARABLE
19 INJECTOR. THIS WILL BE AN OUTPATIENT PROCEDURE
20 BROADLY AVAILABLE THROUGH MEDICARE AND PRIVATE
21 INSURANCE TO THE COMMUNITY OF PATIENTS WITH HEART
22 ATTACKS.

23 "THE REVIEWERS RECOGNIZE THE PROJECT AS
24 UNIQUE; CIRM HAS NOTHING ELSE LIKE IT. FURTHER, THE
25 COMMENTS OF CRITICS WERE RESPONDED TO IN A SERIOUS

BETH C. DRAIN, CA CSR NO. 7152

1 FASHION, SHOWING THE WILLINGNESS TO ADAPT WHEN
2 APPROPRIATE. SOME REVIEWERS ARE EXPERTS AND WERE
3 RESPONDED TO ACCORDINGLY.

4 "FINALLY, BECAUSE OF THE SEEMING EASE AND
5 AFFORDABILITY OF ITS ADMINISTRATION, THIS APPROACH
6 MAY ADVANCE HEALTH JUSTICE SO THAT NOT ONLY THE
7 SUPER RICH CAN AFFORD TO BE MADE WELL. YOUR YES
8 VOTE FOR TRAN1-12907 WILL ALLOW THE FULFILLMENT OF
9 FDA PRE-IND REQUIREMENTS AND PLACE TREATMENT AT THE
10 DOOR OF A FIRST-IN-HUMAN CLINICAL TRIAL. THIS IS
11 EXACTLY WHAT CIRM IS BUILT FOR, TO EASE SUFFERING
12 AND SAVE LIVES AFFORDABLY.

13 "IN THE WORDS OF THE LATE CHRISTOPHER
14 REEVE, WHO, BY THE WAY, DIED OF A HEART ATTACK,
15 PLEASE ALLOW THIS IMPORTANT EFFORT TO GO FORWARD.
16 THANK YOU."

17 CHAIRMAN THOMAS: THANK YOU, KEVIN.

18 MARIA, DO YOU WANT TO JUST INVITE THE NEXT
19 GUEST TO SPEAK HERE?

20 MS. BONNEVILLE: I BELIEVE PILAR IS NEXT.
21 IF YOU'D LIKE TO MAKE A PUBLIC COMMENT, IT'S THREE
22 MINUTES.

23 DR. MCAULIFFE: YES. I'LL SPEAK. I'M
24 MARK MCAULIFFE. I'M A DIRECTOR OF REGENCOR. THAT'S
25 TRAN 12907. I'D LIKE TO SPEAK ON BEHALF OF REGENCOR

BETH C. DRAIN, CA CSR NO. 7152

1 TO THIS COMMITTEE AND TO ENTER INTO THE RECORD THE
2 MERITS OF OUR PROPOSAL THAT WERE VERY ELOQUENTLY
3 STATED JUST A MOMENT AGO.

4 HEART FAILURE, OF COURSE, IS A UBIQUITOUS
5 AND UNMET MEDICAL NEED, THE LARGEST KILLER IN THE
6 STATE, BIGGER THAN CANCER. WE PROPOSED A NEW
7 THERAPEUTIC DERIVED FROM STEM CELL STUDIES THAT
8 PROMOTES HEART REGENERATION. THE TREATMENT WILL BE
9 SIMPLE AND INEXPENSIVE, DELIVERED ON AN OUTPATIENT
10 BASIS, AND, HENCE, READILY AVAILABLE TO UNDERSERVED
11 COMMUNITIES.

12 REVIEWERS WERE EXTREMELY SUPPORTIVE, AS
13 GIL SAMBRANO LAID OUT, PLACING US JUST TWO POINTS
14 BENEATH THE FUNDING PAYLINE. THE SUGGESTED
15 MODIFICATIONS WOULD FACILITATE FDA APPROVAL. WE'RE
16 IN AGREEMENT WITH THESE PERCEPTIVE COMMENTS, AND WE
17 HAVE INCORPORATED THEM INTO OUR WORK PLAN AND WILL
18 WORK WITH CIRM TO MAKE THAT HAPPEN.

19 CIRM SUPPORT IS CRITICAL TO MOVE THIS
20 GAME-CHANGING TECHNOLOGY FORWARD. I THANK YOU VERY
21 MUCH FOR YOUR CONSIDERATION OF OUR PROPOSAL.

22 CHAIRMAN THOMAS: THANK YOU. MARIA.

23 MS. BONNEVILLE: YES. MATTHEW PORTEUS.

24 DR. PORTEUS: HELLO. MY NAME IS MATTHEW
25 PORTEUS, AND I'M THE PI ON TRAN1-12919, THE GENE

BETH C. DRAIN, CA CSR NO. 7152

1 CORRECTION FOR CYSTIC FIBROSIS PROPOSAL. THANK YOU
2 FOR ALLOWING US TO COMMENT.

3 I AM THE PI AND I LED THE DEVELOPMENT OF
4 THE CIRM-SPONSORED FIRST-IN-HUMAN GENE CORRECTION
5 TRIAL FOR SICKLE CELL DISEASE. THIS IS A TEAM THAT
6 HAS PREVIOUSLY BEEN SUCCESSFUL WITH PRIOR CIRM DISC
7 FUNDING AND HAS PUBLISHED TWO PAPERS IN *MOLECULAR*
8 *THERAPY* AND *CELL STEM CELL*.

9 WE ARE GENERALLY PLEASED WITH THE OVERALL
10 ASSESSMENT, AND I AM PARTICULARLY PROUD THAT OUR
11 TEAM HAS DISCOVERED AND IDENTIFIED THAT THERE ARE
12 PATIENTS REPRESENTING UNDERSERVED POPULATIONS IN
13 CALIFORNIA WITH CYSTIC FIBROSIS FOR WHICH THE
14 CURRENT TRIPLE MODULATOR THERAPY SIMPLY WILL NOT BE
15 EFFECTIVE. AND, THUS, OUR PROPOSAL TAKES ON
16 ADDITIONAL SIGNIFICANCE BECAUSE OUR APPROACH WILL
17 TARGET THESE PATIENT POPULATIONS.

18 AS THIS IS A RESUBMISSION AND WE TOOK THE
19 ADVICE TO ADDRESS THE PRIOR COMMENTS FROM THE
20 REVIEWERS, UNFORTUNATELY OUR TEAM AND THE REVIEW
21 TEAM HAVE A FUNDAMENTAL DISAGREEMENT ABOUT THE
22 FEASIBILITY AND NECESSITY OF CERTAIN TYPES OF
23 EXPERIMENTS, WHICH I HAVE OUTLINED IN DETAIL IN THE
24 PUBLIC LETTER.

25 THE TWO MAJOR AREAS OF DISAGREEMENT IS THE

BETH C. DRAIN, CA CSR NO. 7152

1 REVIEWERS' DESIRE, WHICH WE SHARE, ABOUT
2 DEMONSTRATING THE FUNCTIONALITY OF OUR
3 GENE-CORRECTED CELLS IN AN ANIMAL MODEL; THAT IS,
4 HUMAN CELLS FUNCTIONING IN IMMUNODEFICIENT MICE. WE
5 NOTE, THOUGH, THAT IN THE HEMATOPOIETIC SYSTEM,
6 FUNCTIONALITY OF CELLS IS NOT NECESSARY FOR APPROVAL
7 OF AN IND, INSTEAD SIMPLE ENGRAFTMENT IS WHAT IS
8 NEEDED TO BE DEMONSTRATED.

9 THE SECOND MAJOR FLAW IS THAT THE PROPOSED
10 EXPERIMENTS, AND, AGAIN, WE AGREE WITH THE REVIEWERS
11 THAT WE WISH WE COULD DO THEM, ARE SIMPLY NOT
12 TECHNICALLY FEASIBLE. THAT IS, IT WOULD BE
13 IMPOSSIBLE FOR US TO PERFORM THE EXPERIMENTS AND
14 COMPLETE THE MILESTONE IF WE FOLLOWED THE REVIEWERS'
15 SUGGESTIONS.

16 IN OUR LETTER WE PROPOSE A POTENTIAL PATH
17 FORWARD, WHICH IS TO USE THE PROPOSED INTERACT
18 MEETING WITH THE FDA TO NOT ONLY DISCUSS THE PLANNED
19 TUMORIGENICITY AND TOXICOLOGY STUDY FOR A
20 GENETICALLY ENGINEERED CELL PRODUCT, BUT ALSO AS A
21 FORUM TO GET FEEDBACK ON THE NECESSITY OF
22 ESTABLISHING FUNCTIONAL SECRETORY CELLS IN THE
23 RODENT MODEL RATHER THAN JUST SIMPLE ENGRAFTMENT.

24 THANK YOU AGAIN FOR ALLOWING US TO SPEAK,
25 AND WE ARE HOPEFUL THAT THIS PROJECT CAN CONTINUE TO

BETH C. DRAIN, CA CSR NO. 7152

1 BE FUNDED AND SUPPORTED BY THE CIRM PROGRAM.

2 CHAIRMAN THOMAS: THANK YOU, DR. PORTEUS.

3 MS. BONNEVILLE: PAUL, I BELIEVE YOU'RE
4 NEXT. YOU ARE ON MUTE SO WE CAN'T HEAR YOU.

5 MR. BRESGE: MY APOLOGIES. CAN YOU HEAR
6 ME NOW?

7 CHAIRMAN THOMAS: YES.

8 MR. BRESGE: THANK YOU. AND, AGAIN, THANK
9 YOU AS THE MEMBERS OF THE ICOC JUST FOR PROVIDING
10 THIS OPPORTUNITY FOR ME TO TALK ABOUT OUR GRANT
11 12889, OPTOGENETICS THERAPY FOR TREATING RETINITIS
12 PIGMENTOSA AND OTHER IRD'S.

13 WE WERE VERY PLEASED THAT OUR APPLICATION
14 HAD UNANIMOUS VOTES IN FAVOR OF THE SCIENTIFIC
15 RATIONALE AND THE SIGNIFICANCE FOR OUR PROGRAM. AND
16 WE UNDERSTAND THAT THE REVIEWERS HAD SOME CONCERNS
17 ABOUT OUR TIMELINES, WHICH ARE FIXABLE. SO THE FACT
18 THAT WE HAD SUCH A STRONG REVIEW FOR THE SCIENTIFIC
19 RATIONALE, OF COURSE, IS VERY IMPORTANT AND VERY
20 EXCITING TO US.

21 A LONG TIME -- ACTUALLY IT'S A SHORT TIME,
22 IT'S ONLY ABOUT SIX MONTHS, BUT A LONG TIME
23 DEVELOPMENT OF SUCH AN EARLY STAGE PROGRAM. OVER
24 THE LAST MONTHS WE'VE ACHIEVED A VERY SIGNIFICANT
25 MILESTONE OF SELECTING OUR CDMO AND ALSO ENGAGING

BETH C. DRAIN, CA CSR NO. 7152

1 ADDITIONAL GENE THERAPY EXPERTS. AND WE WERE
2 ACTUALLY MODIFYING OUR TIMELINES IN THE TIME THAT WE
3 GOT THE COMMENTS BACK FROM THE REVIEWERS. SO WE
4 COMPLETELY AGREE WITH ALL OF THE REVIEWERS' COMMENTS
5 THAT OUR TIMELINES WERE TOO AMBITIOUS. AND WE DID
6 SEND IN -- CRAWFORD BROWN IS OUR CMC EXPERT, AND I
7 BELIEVE THAT HE'S ON THE LINE AS WELL TODAY. WE DID
8 SEND IN OUR ADJUSTED TIMELINES, WHICH ARE STILL
9 WITHIN THE 30-MONTH PERIOD OF THE GRANT, BUT WE
10 AGREE MUCH MORE REALISTIC CONSIDERING THE WORK THAT
11 HAS TO BE DONE.

12 JUST A COMMENT ABOUT RESUBMITTING AND THE
13 ADDITIONAL TIME. FOR THESE PATIENTS WHO ARE BLIND
14 OR NEARLY BLIND, A DELAY OF SIX MONTHS OR NINE
15 MONTHS OF FUNDING CAN BE VERY, VERY SIGNIFICANT.
16 ALSO, GIVEN THE ENVIRONMENT WITH THE GENE THERAPY
17 SPACE TODAY AND THE AVAILABILITY OF VENDORS, PUSHING
18 OUT TIMELINES COULD CAUSE MORE OF A DELAY THAN
19 SIMPLY THE SIX MONTHS FOR RE-REVIEW. SO WE REALLY
20 ASK THE ICOC TO PLEASE CONSIDER FUNDING OUR
21 APPLICATION. AND I'LL HAND IT OVER TO CRAWFORD
22 BROWN. I THINK THAT HE HAS SOME MORE GRANULAR
23 COMMENTS THAT WILL HELP THE ICOC GET SATISFACTION
24 THAT WE HAVE ADDRESSED ALL OF THE REVIEWERS'
25 CONCERNS. THANK YOU AGAIN.

BETH C. DRAIN, CA CSR NO. 7152

1 DR. BROWN: THANK YOU, PAUL. MY NAME IS
2 CRAWFORD BROWN, AND I WOULD LIKE TO UPDATE YOU WITH
3 REGARD TO RAY-001 CMC PLANS. AS PAUL INDICATED, WE
4 NOW HAVE A FAR MORE TO MATURE PLAN, AND WE HAVE
5 UPDATED IN THIS IN A LETTER SUBMITTED LAST WEEK THAT
6 SHOWS THE MEANS BY WHICH WE CAN DELIVER THIS
7 IMPORTANT NEW MEDICINE TO THE CLINIC AND HAVE
8 REVISED OUR TIMELINES ACCORDINGLY.

9 OVER THE PAST SIX MONTHS, WE'VE COMPLETED
10 INTERNATIONAL DILIGENCE ON THE CDMO ENVIRONMENT TO
11 BE ABLE TO SELECT A PARTNER CAPABLE OF TAKING THIS
12 PRODUCT FORWARD IN EXPEDITED FASHION.

13 WE HAVE ALSO COMPLETED IN-PERSON DUE
14 DILIGENCE WITH IT. THROUGH OUR DISCUSSIONS WITH THE
15 CDMO'S IN TERMS OF THE RAPIDLY CHANGING ENVIRONMENT
16 IN THE MARKETPLACE FOR BIOLOGIC PRODUCTION, IT'S
17 CLEAR THAT WE'VE HAD TO SHIFT OUR TIMELINES BY NINE
18 MONTHS AS REFLECTED IN THE GRANT, AND THIS IS NOTED
19 BY THE REVIEWERS WITH REGARDS TO THE GMP PLASMID
20 TIMELINE WHICH WE'VE UPDATED TO REFLECT THE
21 LEAD-TIME, THE MANUFACTURING, AND RELEASE OF THE
22 PLASMID.

23 HAVING PREVIOUSLY HELD POSITION AS CEO OF
24 A CDMO, AND AS SBT BIOLOGICS R&D IN ALLERGAN
25 ACCOUNTABLE FOR OVER A HUNDRED MILLION U.S. IN

BETH C. DRAIN, CA CSR NO. 7152

1 OUTSOURCING, WE ARE WELL PLACED TO CONFIRM THAT THE
2 CAPACITY CRUNCH IN THIS SECTOR IS EXTREMELY SEVERE
3 AND IS ONLY MADE WORSE BY CAPACITY UTILIZED FOR
4 COVID-19 VACCINE PRODUCTION. THEREFORE, THE DELAY
5 IN THE GRANT IS NOT SIMPLY MEASURED IN DAYS OR
6 WEEKS. BECAUSE CDMO'S ARE BOOKING THE PRODUCTION
7 SLOTS SIX TO TWELVE MONTHS IN ADVANCE, THIS WILL BE
8 A VERY SIGNIFICANT IMPACT IN DELAYING THIS PRODUCT
9 GOING FORWARD FOR PATIENTS. AND, THEREFORE, IT'S
10 REALLY CRITICAL THAT WE BOOK OUR SLOT SO THAT THIS
11 ASSET CAN GO FORWARD. THANK YOU.

12 CHAIRMAN THOMAS: THANK YOU. MARIA, ARE
13 THERE ANY OTHER PUBLIC COMMENTS?

14 MS. BONNEVILLE: I DO NOT SEE ANY HANDS
15 RAISED.

16 CHAIRMAN THOMAS: OKAY. SO THANK YOU ALL
17 THAT GAVE PUBLIC COMMENTS. WE APPRECIATE AS ALWAYS
18 YOU COMING BEFORE US TO SPEAK.

19 THE MOTION WE HAVE AT THE MOMENT IS TO
20 CLOSE OUT THE NOT FOR FUNDING CATEGORY. I WILL ASK
21 THE BOARD IF, HAVING HEARD PUBLIC COMMENTS, ARE
22 THERE ANY -- IS THERE ANY REASON NOT TO VOTE ON THIS
23 MOTION? AND THAT WOULD BE IF NOW A MEMBER OF THE
24 BOARD WOULD LIKE TO MOVE ONE OF THESE PROJECTS BACK
25 UP TO THE FUNDING ROUND. WE HAVE --

BETH C. DRAIN, CA CSR NO. 7152

1 MS. BONNEVILLE: WE HAVE A MOTION ON THE
2 TABLE, SO YOU WOULD HAVE TO CHANGE THAT MOTION.

3 CHAIRMAN THOMAS: OKAY. FAIR ENOUGH. HOW
4 WOULD WE DO THAT, MARIA?

5 MR. MARKS: J.T., THIS IS KEVIN. YOU NEED
6 TO GO FORWARD AND JUST TAKE A VOTE ON THE MOTION
7 THAT'S PENDING.

8 CHAIRMAN THOMAS: OKAY. AND SO --

9 MR. MARKS: AND IF THAT MOTION IS
10 DECLINED, THEN YOU CAN ENTERTAIN A NEW MOTION TO
11 ADVANCE SOMETHING FROM THE UNFUNDED TO THE FUNDED
12 CATEGORY.

13 DR. FISHER: THE ALTERNATIVE WOULD BE
14 SOMEONE COULD OFFER A FRIENDLY AMENDMENT TO THE
15 MOTION THAT COULD BE ACCEPTED BY THE PERSON MAKING
16 THE MOTION.

17 MR. MARKS: CORRECT.

18 DR. FISHER: I DON'T KNOW THAT YOU'D WANT
19 TO VOTE TO APPROVE REMOVING ALL THESE AND THEN HAVE
20 ANOTHER VOTE TO PUT ONE BACK IN. THOSE WOULD
21 CONTRADICT ONE ANOTHER IT WOULD SEEM.

22 CHAIRMAN THOMAS: ADRIANA.

23 DR. PADILLA: YES. THANK YOU. AFTER
24 HEARING EVERYTHING, I REALLY WAS CONFLICTED WITH
25 TRAN 12907. I HAVE A LOT OF PATIENTS THAT HAVE

BETH C. DRAIN, CA CSR NO. 7152

1 SUFFERED HEART ATTACKS AND COMPLICATIONS THEREOF
2 BOTH PHYSICALLY AND EMOTIONALLY. AND I THINK THIS
3 IS A DIFFERENT TYPE OF PROJECT THAT CIRM HAS ON ITS
4 PORTFOLIO. AND I WOULD LIKE TO SUPPORT THAT WE MOVE
5 IT UP. IT HAS A MINORITY REPORT. THE RESEARCH TEAM
6 IS VERY AGREEABLE TO WORK WITH THE GRANTS WORKING
7 GROUP RECOMMENDATION. AND I'D LIKE TO TRY TO MOVE
8 IT UP. I'M NOT SURE HOW WE'RE GOING TO DO IT WITH
9 THE PROPOSAL ON HAND, BUT IF IT CAN HAPPEN, THAT
10 WOULD BE GREAT.

11 CHAIRMAN THOMAS: SO, KEVIN, CAN YOU
12 DESCRIBE THE FRIENDLY AMENDMENT PROCESS, WHICH IS, I
13 BELIEVE, HOW YOU WOULD ADDRESS ADRIANA'S COMMENT?

14 MR. MARKS: SO ADRIANA CAN PROPOSE AN
15 AMENDMENT THAT WOULD HAVE TO THEN BE ACCEPTED BY YOU
16 AS THE MOVANT AS AN AMENDMENT TO THE MOTION THAT'S
17 ON THE FLOOR, AND THEN AL WOULD AGREE AS THE SECOND.

18 DR. PADILLA: SO THEN I WOULD LIKE TO
19 PROPOSE A FRIENDLY AMENDMENT TO THE MOTION TO MOVE
20 TRAN 12907 TO THE FUNDED CATEGORY.

21 CHAIRMAN THOMAS: I WILL ACCEPT THAT
22 AMENDMENT. AL?

23 MR. ROWLETT: I WILL ACCEPT AS THE SECOND.

24 CHAIRMAN THOMAS: OKAY. ARE THERE ANY
25 OTHER FRIENDLY AMENDMENTS THAT ANYBODY WOULD LIKE TO

BETH C. DRAIN, CA CSR NO. 7152

1 OFFER?

2 MS. BONNEVILLE: WE SHOULD VOTE ON THIS
3 FIRST.

4 CHAIRMAN THOMAS: I'M SORRY, MARIA.
5 YOU'VE GOT FEEDBACK THERE.

6 DR. FISHER: I THINK YOU WOULD CALL FOR
7 ANY FURTHER DISCUSSION NOW THAT YOU HAVE A MOTION
8 AND A SECOND.

9 MS. BONNEVILLE: CORRECT.

10 CHAIRMAN THOMAS: THE QUESTION IS DO WE
11 VOTE ON -- KEVIN AND MARIA, DO WE VOTE ON THIS
12 FRIENDLY -- THE FRIENDLY AMENDMENT FIRST, OR DO WE
13 HAVE ANY OTHERS THAT MIGHT BE ADDED AND HAVE AN
14 OMNIBUS VOTE AFTER THAT?

15 MR. MARKS: I THINK THE PREFERRED METHOD
16 AT THIS STAGE, CONSISTENT'S WITH FRED'S PREVIOUS
17 COMMENT OF ONCE THEY'RE OUT, IT'S TOUGH TO BRING
18 THEM BACK, IS THAT IF THERE'S ANY OTHER PROPOSALS TO
19 AMEND A CURRENT MOTION, AN AMENDMENT TO BRING
20 ANYTHING ELSE FORWARD FROM THE UNFUNDED TO FUNDED,
21 WE INCLUDE THAT AND THEN JUST DO IT AS ONE BIG
22 MOTION WITH ALL TACKED-ON AMENDMENTS.

23 CHAIRMAN THOMAS: ARE THERE OTHER FRIENDLY
24 AMENDMENTS THAT ANYBODY WOULD LIKE TO OFFER IN
25 CONNECTION WITH OTHER CURRENTLY UNFUNDED PROJECTS?

BETH C. DRAIN, CA CSR NO. 7152

1 SO I'M GOING TO MOVE THAT, WITH RESPECT TO
2 12889, GENERALLY SPEAKING WHEN PROJECTS, OR I SHOULD
3 SAY MORE OFTEN THAN NOT, WHEN PROJECTS ARE NOT
4 RECOMMENDED FOR FUNDING, IT STARTS WITH THE SCIENCE,
5 WHICH HERE IS NOT SOMETHING THAT'S AN ISSUE. THIS
6 IS ALL ABOUT TIMELINE AND REALISTIC ACHIEVEMENT OF
7 GOALS, WHICH I BELIEVE THAT HAS BEEN ADDRESSED IN
8 THE LETTERS THAT HAVE BEEN SUBMITTED. VERY
9 DIFFICULT TO TELL IF THEY'VE BEEN ADEQUATELY
10 ADDRESSED, ALTHOUGH IT SOUNDS LIKE THERE'S A VERY
11 GOOD-FAITH EFFORT TO DO SO. AND TO THE EXTENT THAT
12 WE WOULD APPROVE SOMETHING LIKE THIS, WOULD WANT TO
13 MAKE IT CLEAR THAT THE PI AND TEAM NEED TO WORK
14 CLOSELY WITH THE CIRM TEAM TO ENSURE THAT THE
15 PROCESS IS ONE THAT HAS A REALISTIC TIMELINE THAT IS
16 DOABLE. BUT I BELIEVE THAT, GIVEN THAT THE SCIENCE
17 IS VERY SOLID HERE, I WOULD MOVE THIS UP. SO I
18 WOULD FRIENDLY AMEND MYSELF AS IT WERE.

19 AL, HOW DOES THAT SOUND TO YOU?

20 MR. ROWLETT: J.T., I'M NOT AS COMPELLED
21 ON THIS PARTICULAR APPLICATION. SO WHILE I, AGAIN,
22 AM IMPACTED BY THE CHAIR'S PASSIONATE APPEAL, I AM
23 GOING TO STAY WITH THE GWG RECOMMENDATION.

24 CHAIRMAN THOMAS: OKAY. TOTALLY FAIR
25 ENOUGH. SO WE HAVE -- ARE THERE ANY OTHER PROJECTS

BETH C. DRAIN, CA CSR NO. 7152

1 BESIDES 907 THAT HAVE A FRIENDLY AMENDMENT? HEARING
2 NONE, KEVIN, PLEASE DESCRIBE FOR US EXACTLY WHAT WE
3 ARE VOTING ON HERE.

4 MR. MARKS: SO WHILE THE ORIGINAL MOTION
5 WAS TO NOT MOVE ANYTHING FROM THE UNFUNDED TO THE
6 FUNDING CATEGORY, THE AMENDMENT WAS TO CHANGE THAT
7 AND OFFER TRAN1-12907 AND MOVE THAT FROM THE
8 UNFUNDED TO THE FUNDED CATEGORY.

9 CHAIRMAN THOMAS: OKAY. YOU'VE NOW ALL
10 HEARD THE MOTION. MARIA, WILL YOU PLEASE CALL THE
11 ROLL.

12 MS. BONNEVILLE: DAN BERNAL. ANNE-MARIE
13 DULIEGE.

14 DR. DULIEGE: NO.

15 MS. BONNEVILLE: YSABEL DURON.

16 MS. DURON: AYE.

17 MS. BONNEVILLE: FRED FISHER.

18 DR. FISHER: AYE.

19 MS. BONNEVILLE: LEONDRA CLARK-HARVEY.
20 DAVID HIGGINS.

21 DR. HIGGINS: YES.

22 MS. BONNEVILLE: RICH LAJARA.

23 MR. LAJARA: YES.

24 MS. BONNEVILLE: DAVE MARTIN.

25 DR. MARTIN: NO.

BETH C. DRAIN, CA CSR NO. 7152

1 MS. BONNEVILLE: LAUREN MILLER-ROGEN.

2 ADRIANA PADILLA.

3 DR. PADILLA: YES.

4 MS. BONNEVILLE: JOE PANETTA.

5 MR. PANETTA: YES.

6 MS. BONNEVILLE: AL ROWLETT.

7 MR. ROWLETT: YES.

8 MS. BONNEVILLE: JONATHAN THOMAS.

9 CHAIRMAN THOMAS: YES.

10 MS. BONNEVILLE: I'M GOING TO NEED A
11 MINUTE TO DO THIS. THE MOTION CARRIES.

12 CHAIRMAN THOMAS: OKAY. THANK YOU. SO
13 NOW WE HAVE THE LAST VOTE, WHICH IS DO WE HAVE A
14 MOTION TO APPROVE ALL PROJECTS IN GREEN THAT ARE
15 RECOMMENDED FOR FUNDING, WHICH NOW INCLUDE 907?

16 DR. FISCHER-COLBRIE: SO MOVED.

17 CHAIRMAN THOMAS: THANK YOU, MARK. IS
18 THERE A SECOND?

19 MS. BONNEVILLE: MARK, YOU CANNOT MAKE THE
20 MOTION ON THIS. SORRY. IT HAS TO BE SOMEONE
21 WITHOUT A CONFLICT.

22 MR. PANETTA: I'LL MAKE THE MOTION.

23 MR. ROWLETT: I'LL SECOND THE MOTION.

24 CHAIRMAN THOMAS: THANK YOU, AL. IS THERE
25 DISCUSSION BY MEMBERS OF THE BOARD? HEARING NONE,

BETH C. DRAIN, CA CSR NO. 7152

1 IS THERE DISCUSSION BY MEMBERS OF THE PUBLIC?

2 MARIA, DO YOU SEE ANY?

3 MS. BONNEVILLE: THERE ARE NO HANDS
4 RAISED.

5 CHAIRMAN THOMAS: HEARING NONE, MARIA,
6 WILL YOU PLEASE CALL THE ROLL.

7 MS. BONNEVILLE: AS A REMINDER, FOR THOSE
8 MEMBERS WITH A CONFLICT, IF YOU CAN SAY EITHER YES
9 OR NO EXCEPT FOR THOSE WITH WHICH I HAVE A CONFLICT.

10 DAN BERNAL. ANNE-MARIE DULIEGE.

11 DR. DULIEGE: NO, AND I DON'T THINK I HAVE
12 A CONFLICT.

13 MS. BONNEVILLE: YSABEL DURON.

14 MS. DURON: AYE.

15 MS. BONNEVILLE: MARK FISCHER-COLBRIE.

16 DR. FISCHER-COLBRIE: YES, EXCEPT FOR
17 THOSE THAT I HAVE A CONFLICT.

18 MS. BONNEVILLE: FRED FISHER.

19 DR. FISHER: YES.

20 MS. BONNEVILLE: ELENA FLOWERS.

21 DR. FLOWERS: YES, EXCEPT THOSE FOR WHICH
22 I HAVE A CONFLICT.

23 MS. BONNEVILLE: LEONDRA CLARK-HARVEY.

24 DAVID HIGGINS.

25 DR. HIGGINS: YES.

BETH C. DRAIN, CA CSR NO. 7152

1 MS. BONNEVILLE: STEVE JUELSGAARD.

2 MR. JUELSGAARD: YES, EXCEPT FOR THOSE
3 WITH WHICH I HAVE A CONFLICT.

4 MS. BONNEVILLE: RICH LAJARA.

5 MR. LAJARA: YES.

6 MS. BONNEVILLE: DAVE MARTIN.

7 DR. MARTIN: YES.

8 MS. BONNEVILLE: CHRISTINE MIASKOWSKI.

9 DR. MIASKOWSKI: YES, EXCEPT FOR THOSE
10 WITH WHICH I HAVE A CONFLICT.

11 MS. BONNEVILLE: LAUREN MILLER-ROGEN.
12 ADRIANA PADILLA.

13 DR. PADILLA: YES.

14 MS. BONNEVILLE: JOE PANETTA.

15 MR. PANETTA: YES.

16 MS. BONNEVILLE: AL ROWLETT.

17 MR. ROWLETT: YES.

18 MS. BONNEVILLE: JONATHAN THOMAS.

19 CHAIRMAN THOMAS: YES.

20 MS. BONNEVILLE: ART TORRES.

21 MR. TORRES: FINALLY, AYE, EXCEPT FOR
22 THOSE WITH WHICH I HAVE A CONFLICT.

23 MS. BONNEVILLE: LOVE HEARING YOUR VOICE,
24 ART. THANK YOU.

25 KAROL WATSON.

BETH C. DRAIN, CA CSR NO. 7152

1 THE MOTION CARRIES.

2 CHAIRMAN THOMAS: THANK YOU, MARIA.

3 ON TO ACTION ITEM NO. 4, CONSIDERATION OF
4 APPLICATIONS SUBMITTED IN RESPONSE TO CLINICAL TRIAL
5 STAGE PROJECTS PROGRAM ANNOUNCEMENT CLINS 1, 2, OR
6 3. PRESENTATION FROM DR. SAMBRANO.

7 DR. SAMBRANO: THANK YOU, MR. CHAIRMAN.
8 LET ME PUT UP MY PRESENTATION. HOPEFULLY EVERYONE
9 CAN SEE THAT.

10 SO THESE ARE THE GWG RECOMMENDATIONS FOR
11 APPLICATIONS THAT WERE SUBMITTED TO THE CLIN
12 PROGRAM. AND A REMINDER THAT THE CLIN PROGRAM HAS
13 THREE DIFFERENT OFFERINGS FOR LATE STAGE PRECLINICAL
14 PROJECTS, WHICH IS THE CLIN1; FOR CLINICAL TRIALS,
15 WHICH IS CLIN2; AND FOR SUPPLEMENTAL ACCELERATING
16 ACTIVITIES, WHICH IS A CLIN3. SO TODAY'S
17 APPLICATION IS A CLIN1 THAT WE WILL BE CONSIDERING.

18 HERE IS AN UPDATE ON THE CLINICAL BUDGET.
19 SO WE HAVE AN ANNUAL ALLOCATION OF 162 MILLION FOR
20 THE FISCAL YEAR 21/22. AND SO THE AMOUNT REQUESTED
21 TODAY FOR THE ONE PROPOSAL IS 4.1 MILLION. THERE
22 ARE 49 MILLION THAT HAVE BEEN APPROVED IN OTHER
23 AWARDS. AND SO THAT IF TODAY'S APPLICATION IS
24 APPROVED, THAT WOULD LEAVE US A BALANCE OF 108.9
25 MILLION.

BETH C. DRAIN, CA CSR NO. 7152

1 SO THE REVIEW CRITERIA, WHICH IS VERY
2 SIMILAR TO WHAT YOU'VE SEEN BEFORE, THE BASIC FIVE
3 QUESTIONS THAT DETERMINE MERIT OF AWARDS: THE
4 SIGNIFICANCE AND POTENTIAL FOR IMPACT, RATIONALE,
5 THE PLAN AND DESIGN, FEASIBILITY, AND ADDRESSING THE
6 NEEDS OF UNDERSERVED COMMUNITIES, ALL ELEMENTS OF
7 WHAT CONTRIBUTES TO THE SCIENTIFIC SCORE.

8 THE SCIENTIFIC SCORE IN THIS CASE IS A
9 SYSTEM OF 1, 2 OR 3. SO THOSE THAT RECEIVE A SCORE
10 OF ONE HAVE EXCEPTIONAL MERIT AND WARRANT FUNDING.
11 A SCORE OF 2 MEANS IT NEEDS IMPROVEMENT, AND THOSE
12 APPLICATIONS TYPICALLY GO BACK TO THE APPLICANT FOR
13 REVISIONS THAT ARE THEN CONSIDERED AGAIN BY THE GWG.
14 A SCORE OF 3 MEANS IT'S SUFFICIENTLY FLAWED THAT IT
15 DOESN'T WARRANT FUNDING, AND THOSE CANNOT BE
16 RESUBMITTED FOR AT LEAST SIX MONTHS.

17 THERE ARE TWO ELEMENTS THAT ARE RELATED TO
18 DIVERSITY WITHIN THE APPLICATIONS. SO JUST WANT TO
19 CALL THEM OUT AND DISTINGUISH THEM. FIRST IS
20 ADDRESSING THE NEEDS OF UNDERSERVED COMMUNITIES,
21 WHICH ALIGNS WITH THE SCIENTIFIC REVIEW CRITERIA NO.
22 5. AND SO THIS SECTION DESCRIBES THE APPLICANT'S
23 PLAN FOR OUTREACH AND ENROLLMENT OF A DIVERSE
24 PATIENT COHORT THAT WOULD ACCOUNT FOR RACIAL,
25 ETHNIC, AND GENDER DIVERSITY. AND SO THIS IS PART

BETH C. DRAIN, CA CSR NO. 7152

1 OF THE OVERALL PROJECT AND INCORPORATED INTO THE
2 SCIENTIFIC MERIT SCORE OF A 1, 2, OR 3. SO IT MEANS
3 THAT WITHIN THAT SCORE THOSE ELEMENTS FROM THE
4 SCIENTIFIC PERSPECTIVE HAVE BEEN INCORPORATED.

5 THERE IS ALSO, HOWEVER, THE DIVERSITY,
6 EQUITY, AND INCLUSION SECTION WHICH DESCRIBES HOW
7 THE APPLICANT TEAM INCORPORATES DIVERSE PERSPECTIVE
8 AND EXPERIENCE TO IMPROVE THE PROJECT OVERALL. AND
9 THIS COULD ALSO ADDRESS THE OVERALL COMMITMENT OF
10 THE TEAM, WHAT THE TEAM'S TRACK RECORD IS IN
11 PROMOTING DIVERSITY, AS WELL AS OVERLAPPING WITH THE
12 ELEMENTS OF THE PLANS FOR ENROLLMENT AND OUTREACH.
13 SO THAT SECTION IS EVALUATED AND SCORED BY OUR
14 PATIENT ADVOCATE AND NURSE MEMBERS OF THE BOARD, AND
15 THAT IS SHOWN IN THE DEI SCORE WHICH IS ON A SCALE
16 OF ZERO TO TEN.

17 SO WITH THAT AS BACKGROUND, THIS
18 PARTICULAR APPLICATION, CLIN1-12865, IS A CAR-T CELL
19 THERAPY FOR B CELL MALIGNANCIES. AND SO MOST
20 SPECIFICALLY, THE INDICATION IS FOR VARIOUS TYPES OF
21 LEUKEMIAS AND LYMPHOMAS. CLL AND ALL ARE THE MAIN
22 TARGETS. THE GOAL IS TO CONDUCT STUDIES THAT WILL
23 ALLOW THEM TO SUBMIT AN IND TO THE FDA AT THE END OF
24 THE AWARD. AND THE FUNDS REQUESTED ARE 4.1 MILLION.
25 THIS APPLICANT ALSO IS PROVIDING CO-FUNDING.

BETH C. DRAIN, CA CSR NO. 7152

1 ALTHOUGH IT ISN'T NECESSARY FOR THIS APPLICANT TO DO
2 SO, THEY ARE PROVIDING CO-FUNDING AT 858,722.

3 A LITTLE BACKGROUND ON B CELL
4 MALIGNANCIES, WHICH INCLUDE LEUKEMIAS AND LYMPHOMAS
5 THAT IN THIS PARTICULAR INSTANCE, THEY'RE LOOKING TO
6 THOSE THAT HAVE A POOR PROGNOSIS OR THAT HAVE
7 SIGNIFICANT IMPACT ON THE QUALITY OF LIFE AND WHERE
8 OTHER THERAPIES MAY HAVE FAILED. THERE ARE A LACK
9 OF TOLERABLE AND DURABLE THERAPIES FOR PATIENTS,
10 PARTICULARLY FOR RECURRING OR REFRACTORY DISEASE,
11 AND THAT REPRESENTS AN UNMET NEED.

12 SO THE PROPOSED THERAPY HERE UTILIZES A
13 TARGET. THIS IS ROR1 WHICH IS A RECEPTOR TYROSINE
14 KINASE FOUND ON CELLS, AND IT IS HIGHLY EXPRESSED
15 WITHIN B CELL MALIGNANCIES AS WELL AS SOLID TUMORS
16 SUCH AS BREAST, LUNG, AND COLON. AND SO IT OFFERS
17 THE POSSIBILITY OF A CURE FOR PATIENTS WITH THESE
18 MALIGNANCIES AND A POSSIBLE EXPANSION TO SOLID
19 TUMORS IN THE FUTURE. AND THE THERAPEUTIC IS A STEM
20 CELL PROJECT BECAUSE THE CANDIDATE ACTS ON CANCER
21 STEM CELLS.

22 THERE ARE A COUPLE OF SIMILAR PROJECTS IN
23 OUR PORTFOLIO, OUR CLINICAL PORTFOLIO. WE HAVE A
24 PHASE 1B/2A CLINICAL TRIAL ALSO TARGETING B CELL
25 MALIGNANCIES. THIS IS A BIOLOGIC, AN MONOCLONAL

BETH C. DRAIN, CA CSR NO. 7152

1 ANTIBODY, THAT TARGETS THE SAME RECEPTOR TYROSINE
2 KINASE THAT IS COMBINED WITH ANOTHER INHIBITOR FOR
3 THE BRUTON TYROSINE KINASE INHIBITOR. AND SO THAT
4 IS THE SAME BROADER TEAM THAT IS LOOKING AT ROR1 IN
5 A DIFFERENT FORMAT.

6 WE HAVE ANOTHER PHASE 1 TRIAL WHICH
7 TARGETS B CELL MALIGNANCIES. THAT ONE IS A CAR-T
8 CELL THERAPY THAT TARGETS A COMBINATION OF CD 19 AND
9 CD 22 ANTIGENS WHICH ARE OBVIOUSLY DIFFERENT FROM
10 THE ROR1 TARGET IN THIS CURRENT PROPOSAL.

11 THE APPLICANTS HAVE HAD PREVIOUS CIRM
12 FUNDING. THEY HAD A TRANSLATIONAL AWARD, WHICH IS
13 NOW CLOSED, THAT WAS THE PRELUDE TO THIS PARTICULAR
14 PROPOSAL, TARGETING HEMATOLOGIC MALIGNANCIES. AND
15 THE GOAL WAS TO CONDUCT A PRE-IND MEETING WHICH WAS
16 DONE AND THE PROJECT WAS SUCCESSFUL IN DOING SO, AND
17 IT ALLOWED THEM TO PREPARE THE APPLICATION THAT IS
18 BEFORE US TODAY.

19 THE OVERALL SUMMARY OF THE GWG
20 RECOMMENDATION IS THAT IT SCORED A 1, MEANING
21 EXCEPTIONAL MERIT AND WARRANTS FUNDING. THERE WERE
22 NINE SCIENTIFIC MEMBERS THAT GAVE IT A SCORE OF 1,
23 THERE WERE FIVE MEMBERS THAT GAVE IT A SCORE OF 2,
24 THERE WERE NO MEMBERS THAT GAVE IT A SCORE OF 3.
25 THE DEI SCORE FROM OUR BOARD MEMBERS ON THE GWG WAS

BETH C. DRAIN, CA CSR NO. 7152

1 A 9.5, AND THE CIRM TEAM RECOMMENDATION CONCURS WITH
2 THAT OF THE GWG RECOMMENDATION TO FUND THIS PROJECT
3 FOR THE AWARD AMOUNT OF 4.1 MILLION. AND SO BACK TO
4 YOU, MR. CHAIRMAN.

5 CHAIRMAN THOMAS: THANK YOU, DR. SAMBRANO.
6 DO WE HAVE A MOTION TO APPROVE THIS GRANT?

7 DR. DULIEGE: AYE. I MOVE IT.

8 CHAIRMAN THOMAS: THANK YOU, ANNEMARIE.
9 IS THERE A SECOND?

10 MS. DURON: SECOND.

11 CHAIRMAN THOMAS: THANK YOU. DISCUSSION
12 BY MEMBERS OF THE BOARD? HEARING NONE, ANY COMMENTS
13 FROM MEMBERS OF THE PUBLIC?

14 MS. BONNEVILLE: THERE ARE NO HANDS
15 RAISED.

16 CHAIRMAN THOMAS: THANK YOU, MARIA.
17 MARIA, WILL YOU THEN PLEASE CALL THE ROLL.

18 MS. BONNEVILLE: DAN BERNAL. ANNE-MARIE
19 DULIEGE.

20 DR. DULIEGE: YES.

21 MS. BONNEVILLE: YSABEL DURON.

22 MS. DURON: YES.

23 MS. BONNEVILLE: MARK FISCHER-COLBRIE.

24 DR. FISCHER-COLBRIE: YES.

25 MS. BONNEVILLE: FRED FISHER.

BETH C. DRAIN, CA CSR NO. 7152

1
2
3
4
5
6
7
8
9
10
11
12
13
14
15
16
17
18
19
20
21
22
23
24
25

DR. FISHER: YES.
MS. BONNEVILLE: ELENA FLOWERS.
DR. FLOWERS: YES.
MS. BONNEVILLE: LEONDRA CLARK-HARVEY.
DAVID HIGGINS.
DR. HIGGINS: YES.
MS. BONNEVILLE: STEVE JUELSGAARD.
MR. JUELSGAARD: YES.
MS. BONNEVILLE: RICH LAJARA.
MR. LAJARA: YES.
MS. BONNEVILLE: DAVE MARTIN.
DR. MARTIN: YES.
MS. BONNEVILLE: CHRISTINE MIASKOWSKI.
DR. MIASKOWSKI: YES.
MS. BONNEVILLE: LAUREN MILLER-ROGEN.
ADRIANA PADILLA.
DR. PADILLA: YES.
MS. BONNEVILLE: JOE PANETTA.
MR. PANETTA: YES.
MS. BONNEVILLE: AL ROWLETT.
MR. ROWLETT: YES.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: KAROL WATSON.
THE MOTION CARRIES.

BETH C. DRAIN, CA CSR NO. 7152

1 CHAIRMAN THOMAS: THANK YOU, MARIA. THAT
2 CONCLUDES THE ACTION ITEMS FROM TODAY'S AGENDA. WE
3 ARE NOW INTO THE GENERAL PUBLIC COMMENT ON ANY TOPIC
4 OF INTEREST. DO WE HAVE ANY PUBLIC COMMENT AT THIS
5 TIME? ANY HANDS, MARIA?

6 MS. BONNEVILLE: NO.

7 CHAIRMAN THOMAS: OKAY. WELL, THANK YOU
8 VERY MUCH. THAT CONCLUDES TODAY'S AGENDA. WE HAVE
9 A BUSY STRETCH COMING UP IN THE NEXT MONTH WITH A
10 NUMBER OF SUBCOMMITTEE MEETINGS AND THE BOARD
11 MEETING ON, MARIA --

12 MS. BONNEVILLE: DECEMBER 14TH.

13 CHAIRMAN THOMAS: -- DECEMBER 14TH.

14 MS. BONNEVILLE: THAT WILL BE AN ALL-DAY
15 MEETING. SO THERE'S A LOT ON THE AGENDA, AND THAT
16 WILL GET POSTED ON DECEMBER 4TH.

17 CHAIRMAN THOMAS: THANK YOU. SO,
18 EVERYBODY, A VERY HAPPY THANKSGIVING TO YOU AND YOUR
19 FAMILIES. AND WE WOULD LOOK FORWARD TO ALL OF THE
20 WORK THAT WE WILL BE DOING IN THE NEXT MONTH,
21 CULMINATING IN THE BOARD MEETING, AT WHICH TIME AT
22 THE BOARD MEETING, AMONG OTHER THINGS, WE WILL BE
23 ENTERTAINING FINAL ADOPTION OF THE NEW STRATEGIC
24 PLAN AND BE OFF TO THE RACES ON NEW ELEMENTS
25 CONTEMPLATED IN THAT. SO THANK YOU, EVERYBODY, VERY

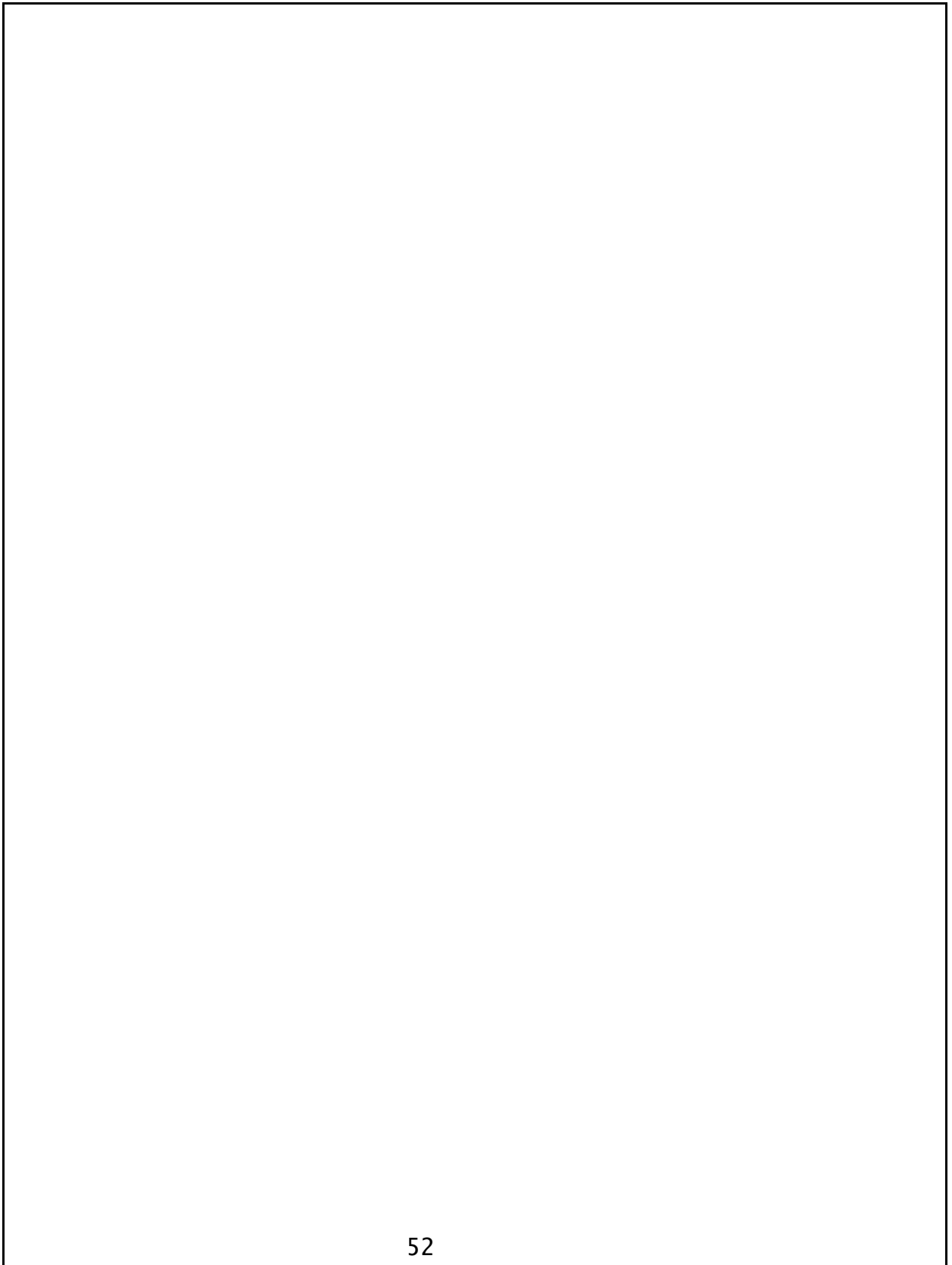
BETH C. DRAIN, CA CSR NO. 7152

1 MUCH FOR ALL OF YOUR HARD WORK. THANK YOU, MARIA,
2 FOR GUIDING THIS AS ALWAYS, KEEPING US GOING ON THE
3 STRAIGHT AND NARROW. AND THANK YOU TO ALL, TO
4 TRICIA AND DOUG AND EVERYBODY ELSE, WHO HELPS MAKE
5 THIS POSSIBLE. HAVE A WONDERFUL HOLIDAY, AND WE
6 WILL SEE YOU SHORTLY THEREAFTER.

7 MS. BONNEVILLE: HAPPY THANKSGIVING,
8 EVERYONE.

9 (THE MEETING WAS THEN CONCLUDED AT
10 11:13 A.M.)

11
12
13
14
15
16
17
18
19
20
21
22
23
24
25



BETH C. DRAIN, CA CSR NO. 7152

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 ZOOM PROCEEDINGS BEFORE THE APPLICATION REVIEW SUBCOMMITTEE OF THE INDEPENDENT CITIZEN'S OVERSIGHT COMMITTEE OF THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE IN THE MATTER OF ITS REGULAR MEETING HELD ON NOVEMBER 23D, 2021, 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) 290-3543