

**BETH C. DRAIN, CA CSR NO. 7152**

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

LOCATION: AS INDICATED ON THE AGENDA

DATE: JULY 19, 2018  
11 A.M.

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

FILE NO.: 2018-12

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**I N D E X**

<b>ITEM DESCRIPTION</b>	<b>PAGE NO.</b>
<b>OPEN SESSION</b>	
1. CALL TO ORDER.	3
2. ROLL CALL.	3
3. READOPTION OF INTELLECTUAL PROPERTY RULES FOR NEW AWARDS?	6
4. CONSIDERATION OF APPLICATIONS SUBMITTED IN RESPONSE TO CLINICAL TRIAL STAGE PROJECTS (CLIN 1, 2 OR 3).	9
5. CONSIDERATION OF APPLICATIONS SUBMITTED IN RESPONSE TO PARTNERING OPPORTUNITY: QUEST AWARDS.	15
<b>CLOSED SESSION</b>	<b>NONE</b>
6. DISCUSSION OF CONFIDENTIAL INTELLECTUAL PROPERTY OR WORK PRODUCT, PREPUBLICATION DATA, FINANCIAL INFORMATION, CONFIDENTIAL SCIENTIFIC RESEARCH OR DATA, AND OTHER PROPRIETARY INFORMATION RELATING TO APPLICATIONS SUBMITTED IN RESPONSE TO AGENDA ITEMS "4" AND "5" ABOVE. (HEALTH & SAFETY CODE 125290.30(F) (3) (B) AND (C)).	
7. PUBLIC COMMENT.	NONE
8. ADJOURNMENT.	88

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JULY 19, 2018; 11 A.M.

CHAIRMAN THOMAS: GOOD MORNING, EVERYBODY,  
AND WELCOME TO THE REGULAR MEETING OF THE ICOC AND  
THE APPLICATION REVIEW SUBCOMMITTEE FOR JULY 2018.  
LIKE TO CALL THE MEETING TO ORDER. MARIA, WILL YOU  
PLEASE CALL THE ROLL.

MS. BONNEVILLE: SURE. GEORGE BLUMENTHAL.  
LINDA BOXER. DAVID BRENNER. KEN BURTIS. DEBORAH  
DEAS.

DR. DEAS: HERE.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: HERE.

MS. BONNEVILLE: JUDY GASSON.

DR. GASSON: HERE.

MS. BONNEVILLE: SAM HAWGOOD. DAVID  
HIGGINS.

DR. HIGGINS: PRESENT.

MS. BONNEVILLE: STEPHEN JUELSGAARD.

MR. JUELSGAARD: HERE.

MS. BONNEVILLE: SHERRY LANSING. LINDA  
MALKAS.

DR. MALKAS: HERE.

MS. BONNEVILLE: DAVE MARTIN.

DR. MARTIN: YES.

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

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1 MS. BONNEVILLE: WE HAVE A QUORUM.

2 CHAIRMAN THOMAS: THANK YOU, MARIA.

3 COUPLE OF MINISTERIAL ITEMS. ONE IS, IF  
4 YOU'RE ON THE LINE AND NOT SPEAKING, IF YOU COULD  
5 PLEASE MUTE YOUR PHONE.

6 THE SECOND IS WITH REGARD TO PUBLIC  
7 COMMENT. MEMBERS OF THE PUBLIC WHO ARE ON THE PHONE  
8 AT A NON-NOTICED MEETING LOCATION WILL HAVE THE  
9 OPPORTUNITY TO DO SO WHEN WE CALL FOR PUBLIC  
10 COMMENT. ONCE WE'VE CALLED FOR PUBLIC COMMENT,  
11 MEMBERS OF THE PUBLIC WILL BE ABLE TO DO SO BY  
12 PRESSING STAR ONE WHICH WILL PLACE YOU IN LINE TO  
13 MAKE YOUR PUBLIC COMMENT. ONCE WE CALL YOUR NAME,  
14 YOU WILL HAVE THREE MINUTES TO MAKE YOUR PUBLIC  
15 COMMENT.

16 THE LAST MINISTERIAL THING I WANT TO POINT  
17 OUT IS FOR THOSE MEMBERS OF THE BOARD ON THE PHONE,  
18 WE E-MAILED OUT A LITTLE WHILE AGO A TABLE WHICH  
19 REFERENCES THE INDICATIONS UNDER CONSIDERATION TODAY  
20 FOR THE QUEST AWARDS AND GIVES INFORMATION ON  
21 EXISTING AWARDS THAT HAVE BEEN MADE IN THE SPACE,  
22 THAT YOU HAVE SOME SORT OF COMPARATIVE ANALYSIS OF  
23 WHAT'S BEEN FUNDED AND WHAT HASN'T. SO YOU MIGHT  
24 WANT TO CHECK THAT E-MAIL. WE HAVE COPIES OF IT  
25 HERE FOR MEMBERS OF THE BOARD WHO ARE PRESENT HERE

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1 IN CIRM HEADQUARTERS AS WELL.

2 OKAY. SO LET'S PROCEED RIGHT IN HERE.  
3 WE'LL GO TO ITEM NO. 3, READOPTION OF INTELLECTUAL  
4 PROPERTY RULES FOR NEW AWARDS. MR. TOCHER.

5 MR. TOCHER: YES. THANK YOU, CHAIRMAN  
6 THOMAS.

7 WITH THIS ITEM, IT'S A BIT IF A FORMALITY.  
8 YOU WILL RECALL THAT A LITTLE EARLIER THIS YEAR WE  
9 ADOPTED FINAL LANGUAGE FOR OUR INTELLECTUAL PROPERTY  
10 RULES THAT WILL BE APPLICABLE TO THE AWARDS.

11 THE REPORTER: THIS IS BETH. I'M SORRY.  
12 THERE IS STILL A LOT OF NOISE ON THE LINE. I CAN  
13 HARDLY HEAR MR. TOCHER.

14 MR. TOCHER: EARLIER THIS YEAR WE ADOPTED  
15 FINAL LANGUAGE TO IMPLEMENT THESE NEW IP POLICY  
16 RULES. AS PART OF THE OFFICE OF ADMINISTRATIVE  
17 LAW'S REVIEW OF THAT FILE, THEY REQUESTED SOME  
18 CHANGES TO NOT THE ACTUAL TEXT OF THE POLICY, BUT  
19 SOME OF THE DOCUMENTS THAT UNDERLIE THE REGULATORY  
20 FILE.

21 AS A CONSEQUENCE, WE CIRCULATED THOSE  
22 REVISED DOCUMENTS TO THE PUBLIC. AND WITH THAT  
23 COMMENT PERIOD, WHICH HAS NOW CONCLUDED, AS A  
24 FORMALITY, THE BOARD MUST THEN READOPT THE IP  
25 POLICY. I NOTE THAT THERE HAS BEEN NO CHANGE,

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1 AGAIN, TO THE ACTUAL TEXT OF THE LANGUAGE THAT THE  
2 BOARD HAS ALREADY APPROVED; BUT THIS IS A STEP  
3 REQUIRED BY THE OFFICE OF ADMINISTRATIVE LAW JUST TO  
4 CLOSE OUT THE PROCESS. AND I WOULD EXPECT THAT WE  
5 WOULD HAVE THIS ALL WRAPPED UP BY THE END OF THIS  
6 MONTH ACCORDING TO MY CONVERSATIONS WITH OAL.

7 SO HAPPY TO TAKE ANY QUESTIONS OR REQUEST  
8 A MOTION TO ADOPT.

9 MR. JUELSGAARD: I MOVE THAT WE --

10 MR. TORRES: SO MOVED.

11 DR. JUELSGAARD: -- ADOPT INTELLECTUAL  
12 PROPERTY RULES.

13 MR. TOCHER: STEVE AND ART IS THE SECOND.

14 MS. WINOKUR: SECOND.

15 CHAIRMAN THOMAS: DO WE HAVE ANY COMMENT  
16 ON THIS ITEM FROM MEMBERS OF THE PUBLIC EITHER HERE  
17 AT HEADQUARTERS OR AT ANY OF THE NOTICED OR  
18 NON-NOTICED LOCATIONS?

19 HEARING NONE, I BELIEVE WE CAN DO A VOICE  
20 VOTE HERE AND CALL THE ROLL FOR MEMBERS ON THE  
21 PHONE.

22 DR. PRIETO: MR. CHAIRMAN, THIS IS  
23 FRANCISCO PRIETO. I JUST WANTED TO SAY I'M ON THE  
24 LINE.

25 CHAIRMAN THOMAS: THANK YOU, FRANCISCO.

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1 ALL THOSE HERE IN PERSON PLEASE SAY AYE,  
2 IN FAVOR SAY AYE. OPPOSED? IT'S UNANIMOUS HERE.  
3 MARIA, WILL YOU PLEASE CALL THE ROLL.

4 MS. BONNEVILLE: DEBORAH DEAS.

5 DR. DEAS: AYE.

6 MS. BONNEVILLE: ANNE-MARIE DULIEGE.

7 DR. DULIEGE: AYE.

8 MS. BONNEVILLE: JUDY GASSON.

9 DR. GASSON: AYE.

10 MS. BONNEVILLE: DAVID HIGGINS.

11 DR. HIGGINS: YES.

12 MS. BONNEVILLE: STEVE JUELSGAARD.

13 MR. JUELSGAARD: YES.

14 MS. BONNEVILLE: LINDA MALKAS.

15 DR. MALKAS: YES.

16 MS. BONNEVILLE: DAVE MARTIN.

17 DR. MARTIN: AYE.

18 MS. BONNEVILLE: SHLOMO MELMED.

19 DR. MELMED: YES.

20 MS. BONNEVILLE: LAUREN MILLER.

21 MS. MILLER: YES.

22 MS. BONNEVILLE: ADRIANA PADILLA.

23 DR. PADILLA: AYE.

24 MS. BONNEVILLE: FRANCISCO PRIETO.

25 DR. PRIETO: AYE.



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1 MS. BONNEVILLE: ROBERT QUINT.  
2 DR. QUINT: YES.  
3 MS. BONNEVILLE: AL ROWLETT.  
4 MR. ROWLETT: YES.  
5 MS. BONNEVILLE: SUZANNE SANDMEYER.  
6 DR. SANDMEYER: YES.  
7 MS. BONNEVILLE: JEFF SHEEHY.  
8 MR. SHEEHY: YES.  
9 MS. BONNEVILLE: OS STEWARD.  
10 DR. STEWARD: YES.  
11 MS. BONNEVILLE: JONATHAN THOMAS.  
12 CHAIRMAN THOMAS: YES.  
13 MS. BONNEVILLE: ART TORRES.  
14 MR. TORRES: AFFIRMATIVE.  
15 MS. BONNEVILLE: KRISTINA VUORI.  
16 DR. VUORI: YES.  
17 MS. BONNEVILLE: DIANE WINOKUR.  
18 MS. WINOKUR: YES.  
19 MS. BONNEVILLE: MOTION CARRIES. THANK  
20 YOU.  
21 CHAIRMAN THOMAS: THANK YOU, MARIA.  
22 ON TO ITEM 4, CONSIDERATION OF  
23 APPLICATIONS SUBMITTED IN RESPONSE TO CLINICAL TRIAL  
24 STAGE PROJECTS CLIN1, 2, OR 3. WE'LL HAVE DR.  
25 SAMBRANO DO A PRESENTATION. OH, DR. PATEL WILL DO

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1 THE PRESENTATION, AND THEN WE'LL TURN THE DISCUSSION  
2 OVER TO MR. SHEEHY.

3 DR. PATEL: THANK YOU, J.T. SO I'LL BE  
4 PRESENTING THE CLINICAL PROGRAM APPLICATIONS TO YOU  
5 TODAY, AND I THANK YOU FOR THE OPPORTUNITY.

6 JUST A REMINDER THAT WE ACTUALLY HAVE  
7 THREE DIFFERENT FUNDING OPPORTUNITIES WITHIN THE  
8 CLINICAL PROGRAM. TODAY WE HAVE ONE APPLICATION FOR  
9 YOUR CONSIDERATION, THE CLIN1 OPPORTUNITY, WHICH IS  
10 IND-ENABLING STUDIES.

11 JUST A REMINDER OF THE GWG SCORING  
12 MECHANISM FOR OUR CLINICAL PROGRAM. A SCORE OF 1  
13 INDICATES EXCEPTIONAL MERIT AND WARRANTS FUNDING; A  
14 SCORE OF 2 WOULD INDICATE THAT IT NEEDS IMPROVEMENT  
15 AND HAS OPPORTUNITY FOR RESUBMISSION; AND A SCORE OF  
16 3 WOULD INDICATE THAT THE APPLICATION IS  
17 SUFFICIENTLY FLAWED AND SHOULD NOT BE RESUBMITTED  
18 FOR SIX MONTHS AFTER REVIEW.

19 SO TO GIVE YOU A BRIEF OVERVIEW OF WHAT  
20 THE STATUS LOOKS LIKE FOR THE CLINICAL PROGRAM AT  
21 THE MOMENT. THE PROGRAM WAS ALLOCATED \$130 MILLION  
22 FOR THE YEAR. 82 MILLION HAVE BEEN APPROVED FOR  
23 SPENDING BY THE BOARD SO FAR THIS YEAR. THE CURRENT  
24 APPLICATION UP FOR REVIEW TODAY IS \$4 MILLION. IF  
25 THAT WERE TO BE APPROVED, THEN THERE WOULD BE \$44

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1 MILLION REMAINING TO FUND THE REST OF THE PROJECTS  
2 FOR THIS CALENDAR YEAR.

3 NOW, AS YOU KNOW, THERE'S A BIG SIX GOALS  
4 THAT CIRM HAS, AND WE ALLOCATED THE FOLLOWING  
5 TARGETS TOWARD THAT BIG SIX GOAL. THERE WERE 12  
6 CLIN2S THAT WERE TARGETED FOR 2018 AND FOUR CLIN1S  
7 FOR 2018. SO FAR THIS YEAR THE BOARD HAS FUNDED SIX  
8 CLIN2 AWARDS, AND THEY FUNDED THREE CLIN1 AWARDS.  
9 IF YOU FUND THE CLIN1 AWARD TODAY, THAT WOULD MAKE  
10 IT FOUR FOR THE YEAR.

11 NOW I'M GOING TO GO INTO THE APPLICATION  
12 ITSELF. THE APPLICATION IS CLIN1-10999. THIS IS A  
13 LATE STAGE PRECLINICAL THERAPY STUDY FOR A THERAPY  
14 FOR PROSTATE CANCER. THE THERAPY ITSELF IS CAR-T  
15 CELLS, AND THESE ARE STEM MEMORY T CELLS THAT  
16 COMPOSE THIS PARTICULAR PRODUCT.

17 THE INDICATION IS FOR PATIENTS WITH  
18 METASTATIC CASTRATE RESISTANT PROSTATE CANCER. THIS  
19 IS THE MOST ADVANCED FORM OF THIS PARTICULAR  
20 DISEASE. THE GOAL FOR THIS PARTICULAR PROJECT IS TO  
21 CONDUCT PRODUCT MANUFACTURING, CONDUCT THE  
22 PRECLINICAL SAFETY AND EFFICACY STUDIES, AS WELL AS  
23 PREPARE AND SUBMIT THE IND. AND THEY'RE REQUESTING  
24 ROUGHLY \$4 MILLION FOR CIRM FUNDING, AND THEY'RE  
25 GOING TO BE CO-FUNDING \$1 MILLION.

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1 TO GIVE YOU AN IDEA ABOUT THIS PARTICULAR  
2 PROJECT, WE'VE PREPARED A FEW ITEMS. FIRST OF ALL,  
3 THE POTENTIAL IMPACT FOR THIS THERAPY. THERE ARE  
4 165,000 NEW CASES OF PROSTATE CANCER SUBMITTED FOR  
5 THIS PARTICULAR YEAR, AND ROUGHLY 30,000 PROSTATE  
6 CANCER DEATHS FOR 2018 AS ESTIMATED BY THE NIH.

7 NOW, THE PARTICULAR DISEASE INDICATION  
8 THAT THIS PRODUCT IS GOING FOR, WHICH IS THE  
9 METASTATIC CASTRATE RESISTANT PROSTATE CANCER  
10 POPULATION, THAT REPRESENTS A VERY SMALL PERCENTAGE  
11 OF THE OVERALL PROSTATE CANCER POPULATION, BUT AN  
12 OVERWHELMING MAJORITY OF THE DEATHS FOR THIS  
13 PARTICULAR DISEASE. IF SUCCESSFUL, THIS PARTICULAR  
14 THERAPY WOULD TARGET THAT MOST NEEDY, HIGHEST RISK  
15 PATIENT POPULATION.

16 IN TERMS OF THE VALUE PROPOSITION, OVER  
17 THE LAST DECADE OR SO, THERE HAS BEEN A STEADY  
18 IMPROVEMENT IN THE STANDARD OF CARE FOR PROSTATE  
19 CANCER. CURRENTLY AVAILABLE THERAPIES FOR PROSTATE  
20 CANCER FOR METASTATIC CASTRATE RESISTANT PROSTATE  
21 CANCER INCLUDES HORMONE THERAPY, CHEMOTHERAPY, AS  
22 WELL AS AN AUTOLOGOUS T-CELL PRODUCT, WHICH IS AN  
23 ACTIVATED T-CELL PRODUCT, ALL OF WHICH HAVE SHOWN  
24 MODEST SURVIVAL BENEFIT IN CLINICAL TRIALS.

25 THE PROPOSED THERAPY HAS A POTENTIAL TO

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1 INDUCE LONG-TERM REMISSION WITH POSSIBLY A SINGLE  
2 TREATMENT.

3 IN TERMS OF WHY THIS IS A STEM CELL  
4 PROJECT, AS I MENTIONED EARLIER, THIS PARTICULAR  
5 PRODUCT IS COMPOSED OF MEMORY STEM T-CELLS.

6 TO GIVE YOU A BRIEF OVERVIEW OF THE  
7 CURRENT PORTFOLIO FOR CIRM WITH RESPECT TO RELATED  
8 PROJECTS, WE ARE NOT CURRENTLY FUNDING ANY PROSTATE  
9 CANCER-DIRECTED PROJECTS, BUT WE DO HAVE SOME SOLID  
10 TUMOR PROJECTS, INCLUDING THERAPIES INCLUDING  
11 THERAPIES OF CELL-DERIVED THERAPIES AS WELL AS SMALL  
12 MOLECULE ANTIBODY-BASED THERAPIES, ALL IN EARLY  
13 PHASE OR IND-ENABLING STUDIES.

14 THIS PARTICULAR APPLICANT HAS RECEIVED  
15 CIRM FUNDING BEFORE. THEY ARE CURRENTLY CONDUCTING  
16 A PHASE 1 TRIAL FOR A CAR-T BASED THERAPY FOR  
17 MULTIPLE MYELOMA, WHICH IS FUNDED BY CIRM.

18 LASTLY, THE GWG REVIEWED THIS APPLICATION  
19 AND UNANIMOUSLY GAVE IT A TIER I RECOMMENDATION.  
20 THERE WERE 12 SCORES FOR TIER I, ZERO SCORES FOR  
21 TIER II, AND ZERO SCORES FOR TIER III. THE CIRM  
22 TEAM RECOMMENDATION FOLLOWS THE GWG RECOMMENDATION  
23 FOR THE AWARD AMOUNT OF \$3,992,090. CHAIRMAN  
24 THOMAS.

25 CHAIRMAN THOMAS: THANK YOU, DR. PATEL.

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1 WE'LL TURN THE DISCUSSION OVER HERE TO MR. SHEEHY.

2 MR. SHEEHY: SO DO I HAVE A MOTION TO  
3 EITHER ACCEPT OR REJECT THE TEAM RECOMMENDATION?

4 MR. TORRES: SO MOVED.

5 MR. JUELSGAARD: SECOND.

6 MR. SHEEHY: TO ACCEPT?

7 MR. TORRES: YES.

8 MR. SHEEHY: IS THERE ANY DISCUSSION? IS  
9 THERE ANY PUBLIC COMMENT AT ANY OF THE SITES? COULD  
10 WE CALL THE ROLL PLEASE.

11 MS. BONNEVILLE: ANNE-MARIE DULIEGE.

12 DR. DULIEGE: YES.

13 MS. BONNEVILLE: DAVID HIGGINS.

14 DR. HIGGINS: YES.

15 MS. BONNEVILLE: STEVE JUELSGAARD.

16 MR. JUELSGAARD: YES.

17 MS. BONNEVILLE: DAVE MARTIN.

18 DR. MARTIN: YES.

19 MS. BONNEVILLE: LAUREN MILLER.

20 MS. MILLER: YES.

21 MS. BONNEVILLE: ADRIANA PADILLA.

22 DR. PADILLA: YES.

23 MS. BONNEVILLE: FRANCISCO PRIETO.

24 DR. PRIETO: AYE.

25 MS. BONNEVILLE: ROBERT QUINT.

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DR. QUINT: YES.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: YES.

MS. BONNEVILLE: JEFF SHEEHY.

MR. SHEEHY: YES.

MS. BONNEVILLE: OS STEWARD.

DR. STEWARD: YES.

MS. BONNEVILLE: JONATHAN THOMAS.

CHAIRMAN THOMAS: YES.

MS. BONNEVILLE: ART TORRES.

MR. TORRES: AYE.

MS. BONNEVILLE: DIANE WINOKUR.

MS. WINOKUR: YES.

MS. BONNEVILLE: THANK YOU. THE MOTION  
CARRIES.

MR. SHEEHY: THANK YOU. I THINK WE'RE  
READY TO ROLL OVER TO THE QUEST AWARDS.

NOW, BEFORE DR. SAMBRANO STARTS, I THINK  
THAT THERE IS A SIGNIFICANT FEATURE OF THIS ROUND IN  
THAT WE HAVE 14 AWARDS IN THE FUNDABLE CATEGORY, BUT  
WE ONLY HAVE FUNDING FOR APPROXIMATELY HALF OF  
THOSE. SO THE DISCUSSION THAT WE RECENTLY HAD ABOUT  
PROGRAMMATIC REVIEW, I THINK, IS NOW REAL. AND THE  
WAY WE WILL PROCEED, I THINK, IS THAT DR. SAMBRANO  
WILL GIVE US AN INTRODUCTION. WE'LL HAVE A

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1 CONVERSATION ABOUT THE GRANTS IN THE NOT FUNDABLE  
2 CATEGORY SO THAT WE CAN DISPOSE OF THOSE. AND THEN  
3 PERHAPS BEFORE WE START LOOKING AT THE FUNDABLE  
4 RANGE, WE MIGHT HAVE A LITTLE BIT OF A BRIEF  
5 DISCUSSION OF SOME OF THE PROGRAMMATIC  
6 CONSIDERATIONS THAT WE MAY WANT TO BRING TO BEAR IN  
7 LOOKING AT MAKING DECISIONS ABOUT THESE  
8 APPLICATIONS.

9 I WILL NOTE, AND I THINK CHAIRMAN THOMAS  
10 NOTED THIS, THERE'S A NICE -- THE TEAM HAS PUT  
11 TOGETHER A NICE SHEET KIND OF GIVING US WHAT WE  
12 FUNDED AND WHAT TYPES OF TECHNOLOGIES ARE INVOLVED  
13 IN THESE APPLICATIONS AND WHAT THE DISEASE TARGETS  
14 ARE. AND WE MIGHT WANT TO LOOK AT THAT AS WELL AS  
15 WE PREPARE FOR THIS DISCUSSION BECAUSE WE'RE GOING  
16 TO HAVE TO MAKE SOME HARD CHOICES TODAY AND I THINK  
17 THE BEGINNING OF MAYBE A TIME WHERE THAT BECOMES  
18 MORE COMMON. DR. SAMBRANO.

19 DR. SAMBRANO: THANK YOU, MR. SHEEHY.  
20 JUST TO START OFF, I WANT TO GIVE YOU AN OVERVIEW ON  
21 THE QUEST PROGRAM AND WHERE IT FITS UNDER OUR  
22 FUNDING OPPORTUNITIES.

23 SO QUEST IS THE HALLMARK OF THE DISCOVERY  
24 PROGRAM WHICH IS INTENDED TO DEVELOP CANDIDATES THAT  
25 CAN LATER BE TRANSLATED AND THEN ULTIMATELY GO TO



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1 THE CLINIC OR BE AVAILABLE TO PATIENTS. SO THE  
2 DISCOVERY PROGRAM USUALLY HAS ONE OR TWO  
3 OPPORTUNITIES PER YEAR. FOR THE QUEST PROGRAM WE  
4 ANTICIPATE HAVING ONE THIS YEAR IN WHICH, AS  
5 MENTIONED, WE ALLOCATED \$10 MILLION AND PROBABLY THE  
6 SAME NEXT YEAR.

7 THE OBJECTIVE OF THE PROGRAM, OF COURSE,  
8 IS TO PROMOTE DISCOVERY OF PROMISING NEW STEM  
9 CELL-BASED TECHNOLOGIES THAT WOULD BE READY FOR  
10 TRANSLATION IN TWO YEARS. SO THIS IS A TWO-YEAR  
11 ENDEAVOR FOR ANYBODY WHO RECEIVES THIS AWARD. AND  
12 THE GOAL IS TO SUPPORT PROJECTS THAT CAN BE  
13 THERAPEUTIC, BUT ALSO A DIAGNOSTIC, A MEDICAL  
14 DEVICE, OR A TOOL THAT CAN BE COMMERCIALIZED.

15 SO MORE SPECIFICALLY ON WHAT QUALIFIES,  
16 THIS IS A PRETTY BROAD SCOPE IN TERMS OF THE  
17 PROJECTS THAT CAN COME IN. IT CAN INCLUDE STEM OR  
18 PROGENITOR CELL THERAPIES, REPROGRAMMED CELL  
19 THERAPIES, SMALL MOLECULES OR BIOLOGICS THAT  
20 STIMULATE OR ACT ON ENDOGENOUS STEM CELLS OR CANCER  
21 STEM CELLS, AND DEVICES, DIAGNOSTIC, OR TOOLS THAT  
22 IN SOME WAY USE STEM PROGENITOR CELLS OR THAT  
23 ADDRESSES A BOTTLENECK IN THE STEM CELL THERAPY  
24 FIELD. SO THAT'S JUST A BIG PICTURE OF THE TYPES OF  
25 PROJECTS THAT WE ACCEPT FOR THIS.

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1 THE GRANTS WORKING GROUP, AS YOU KNOW,  
2 DOES THE SCIENTIFIC MERIT ASSESSMENT OF THESE  
3 APPLICATIONS, AND THEY USE FOUR CRITERIA TO DO THAT,  
4 WHICH INCLUDE WHETHER THE PROJECT HOLDS THE  
5 NECESSARY SIGNIFICANCE AND POTENTIAL FOR IMPACT;  
6 THAT IS, WHAT VALUE IS IT BRINGING TO THE TABLE AND  
7 DOES IT ADDRESS UNMET MEDICAL NEED? IS THE  
8 RATIONALE SOUND, MEANING DOES IT MAKE SENSE AND DO  
9 THEY HAVE DATA TO SUPPORT WHAT THEY INTEND TO DO?  
10 IS THE PROJECT WELL-PLANNED AND DESIGNED? AND IS IT  
11 FEASIBLE, INCLUDING WHETHER THEY HAVE AN APPROPRIATE  
12 TEAM ASSEMBLED AND THE RESOURCES TO CARRY OUT WHAT'S  
13 PROPOSED.

14 IN THIS PARTICULAR TYPE OF COMPETITION,  
15 UNLIKE THE CLINICAL PROGRAM, THE SCORING SYSTEM IS  
16 BASED ON A ONE-TO-A-HUNDRED SCALE. SO APPLICATIONS  
17 THAT RECEIVE A SCORE BETWEEN 85 AND A HUNDRED, THIS  
18 IS BASED ON THE MEDIAN SCORE BY THE SCIENTIFIC  
19 REVIEWERS, WOULD GET A RECOMMENDATION FOR FUNDING.  
20 THOSE THAT SCORE BELOW 85 ARE NOT RECOMMENDED FOR  
21 FUNDING.

22 SO FOR THIS PARTICULAR CYCLE, WE HAD ABOUT  
23 41 OR 43 APPLICATIONS COMING IN. THERE WERE 14  
24 APPLICATIONS THAT WERE RECOMMENDED, AS MR. SHEEHY  
25 MENTIONED EARLIER, AND THE TOTAL REQUEST FROM THOSE

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1 14 APPLICATIONS TOTALS TO JUST OVER \$19 MILLION.  
2 AND AS ALSO NOTED, WE HAVE AVAILABLE TO US 10  
3 MILLION. SO THAT, IN ESSENCE, MEANS WITH ANY  
4 COMBINATION OF THOSE THAT ARE IN THAT TOP TIER, YOU  
5 COULD COVER WITH 10 MILLION AS LITTLE SIX AND AS  
6 MANY AS EIGHT, BUT PRETTY MUCH MOST COMBINATIONS  
7 GIVE YOU SEVEN APPLICATIONS.

8 SO THE CIRM TEAM, AFTER QUITE A BIT OF  
9 DISCUSSION OVER THESE, KNOWING THAT THIS IS  
10 OBVIOUSLY A CHALLENGE FOR ALL OF US IN TERMS OF WHAT  
11 WE CAN FUND, DEVELOPED A RECOMMENDATION. AND I  
12 THINK YOU SHOULD LOOK AT THIS AS A STARTING POINT,  
13 PERHAPS, FOR DISCUSSION. AND SO OUR RECOMMENDATION  
14 AND SUGGESTION TO YOU IS TO FUND THE TOP SEVEN  
15 RANKING APPLICATIONS AS SHOWN IN THE CHART THAT WE  
16 PROVIDED THAT BEGINS WITH THIS DISC2-11131 DOWN TO  
17 DISC2-11175.

18 THIS WOULD UTILIZE 9.4 MILLION OUT OF THE  
19 10 MILLION AVAILABLE IN THE BUDGET. IT CAPTURES THE  
20 FOUR APPLICATIONS THAT RECEIVED A UNANIMOUS VOTE  
21 FROM THE GRANTS WORKING GROUP. THESE SEVEN INCLUDE  
22 SIX CELL THERAPY AND ONE BIOLOGIC APPROACH, WHICH WE  
23 FELT, AMONG THE DIFFERENT APPROACHES, WAS CORE TO  
24 WHAT CIRM DOES AND OVERALL MISSION. IT CAPTURES  
25 FOUR OF FIVE APPLICATIONS WITHIN THAT TOP TIER THAT

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1 HAVE HAD PREVIOUS CIRM FUNDING AND THAT HAS  
2 CONTRIBUTED TO THE DEVELOPMENT OF THE PROJECTS. SO  
3 THAT IS OUR RECOMMENDATION.

4 I'LL JUST NOTE THAT IT DOESN'T NECESSARILY  
5 GIVE PRIORITY TO ANY DISEASE INDICATION OR WHETHER  
6 OR NOT IT WAS REPRESENTED IN OUR PORTFOLIO. BUT  
7 FOLLOWING DISCUSSION, I WILL GIVE AN OVERVIEW OF  
8 EACH OF THE APPLICATIONS THAT INCLUDE SOME OF THESE  
9 ELEMENTS TO GIVE YOU A SENSE OF EACH ONE. MR.  
10 SHEEHY.

11 MR. SHEEHY: JUST ONE QUICK QUESTION  
12 BEFORE WE START IN. DO WE KNOW WHICH ARE THE FIVE  
13 APPLICATIONS WITH PREVIOUS CIRM FUNDING?

14 DR. SAMBRANO: WE DO. SO IN THE CHART,  
15 THE PREVIOUS CIRM FUNDING HAS A COLUMN THAT'S YES OR  
16 NO. AND I CAN JUST TELL YOU THOSE ARE THE FIRST  
17 TWO, WHICH ARE 11131 FOR THE DANON DISEASE  
18 INDICATION. 11157, WHICH IS A CANCER INDICATION.  
19 IT IS 11105 FOR BLADDER CANCER, AND THEN 11175 FOR  
20 TYPE 1 DIABETES. AND THOSE ARE AMONG THE ONES THAT  
21 WE ARE RECOMMENDING, BUT ALSO THERE IS 11199 FOR  
22 HEARING LOSS.

23 MR. SHEEHY: THANK YOU. SO, FIRST, I  
24 THINK, JUST FOR SIMPLICITY, IS THERE ANY DESIRE BY  
25 ANY MEMBER TO MOVE ANY APPLICATION FROM TIER II INTO

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1 TIER I? OKAY. NOT HEARING THAT, COULD I GET A  
2 MOTION TO NOT FUND THE APPLICATIONS IN TIER II?

3 MR. ROWLETT: SO MOVED.

4 MR. SHEEHY: SO AL. STEVE JUELSGAARD AS  
5 THE SECOND.

6 DISCUSSION ABOUT THAT? ANY PUBLIC COMMENT  
7 AT ANY OF THE SITES? SO CAN WE CALL THE ROLL?

8 MS. BONNEVILLE: ANNE-MARIE DULIEGE.

9 DR. DULIEGE: AYE.

10 MS. BONNEVILLE: DAVID HIGGINS.

11 DR. HIGGINS: YES.

12 MS. BONNEVILLE: STEVE JUELSGAARD.

13 MR. JUELSGAARD: YES.

14 MS. BONNEVILLE: DAVE MARTIN.

15 DR. MARTIN: AYE.

16 MS. BONNEVILLE: LAUREN MILLER.

17 MS. MILLER: YES.

18 MS. BONNEVILLE: ADRIANA PADILLA.

19 DR. PADILLA: YES.

20 MS. BONNEVILLE: FRANCISCO PRIETO.

21 DR. PRIETO: AYE.

22 MS. BONNEVILLE: ROBERT QUINT. AL  
23 ROWLETT.

24 MR. ROWLETT: YES.

25 MS. BONNEVILLE: JEFF SHEEHY.

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1 MR. SHEEHY: YES.  
2 MS. BONNEVILLE: JONATHAN THOMAS.  
3 CHAIRMAN THOMAS: YES.  
4 MS. BONNEVILLE: OS STEWARD.  
5 DR. STEWARD: YES, EXCEPT FOR THOSE WITH  
6 WHICH I'M IN CONFLICT.  
7 MS. BONNEVILLE: ART TORRES.  
8 MR. TORRES: AYE.  
9 MS. BONNEVILLE: DIANE WINOKUR.  
10 MS. WINOKUR: YES.  
11 MS. BONNEVILLE: DR. QUINT.  
12 MOTION CARRIES.  
13 MR. SHEEHY: GREAT. THANK YOU.  
14 DR. STEWARD, YOU NO LONGER HAVE CONFLICTS,  
15 SO THERE'S NO IMPEDIMENT TO YOU FULLY PARTICIPATING  
16 IN THE DISCUSSION AS WE GO FORWARD. SO I WANTED TO  
17 MAKE SURE YOU ARE AWARE OF THAT.  
18 DR. STEWARD: YES. THANK YOU VERY MUCH.  
19 MR. SHEEHY: SURE. NOW, IS THERE ANY  
20 DESIRE TO HAVE ANY DISCUSSION BEFORE WE -- ABOUT  
21 PROGRAMMATIC REVIEW BEFORE WE START INTO THE  
22 APPLICATIONS? MR. JUELGAARD.  
23 DR. JUELGAARD: THERE'S A DESIRE ON MY  
24 PART. I'M SURE EVERYBODY WILL RECALL AT THE ICOC  
25 MEETING THAT WE JUST HAD A MONTH OR SO AGO WE

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1 INTRODUCED THE WHOLE NOTION OF PROGRAMMATIC REVIEW.  
2 THIS IS SOMETHING WE HAVEN'T USED FOR SEVERAL YEARS  
3 NOW. I REMEMBER WHEN I FIRST JOINED THE ICOC, WE  
4 ACTUALLY DID ENGAGE IN PROGRAMMATIC REVIEW AT THAT  
5 TIME, BUT SINCE THEN IT'S SORT OF GONE BY THE  
6 WAYSIDE. BUT I THINK IT'S AT THIS POINT APPROPRIATE  
7 TO CONSIDER WHETHER WE WANT TO APPLY PROGRAMMATIC  
8 REVIEW FOR THE APPLICATIONS THAT WERE BROUGHT TO US.

9 SO I NOTE THAT THE STAFF'S RECOMMENDATION  
10 IS BASICALLY BASED ON SCIENTIFIC MERIT AND FUNDING  
11 THOSE IN RANK ORDER. I HAVE A LITTLE DIFFERENT TAKE  
12 ON THAT ASPECT. FROM MY POINT OF VIEW, ONCE AN  
13 APPLICATION HAS RECEIVED A SCORE OF 85 OR GREATER, I  
14 BEGIN TO LOOK LESS AT SCIENTIFIC MERIT BECAUSE, FROM  
15 MY POINT OF VIEW, THEY'VE ALL BEEN DEEMED WORTHY OF  
16 BEING FUNDED. AND IF WE HAD THE MONEY AVAILABLE TO  
17 US RIGHT HERE AND NOW, I WOULD DARE SAY WE WOULD  
18 PROBABLY APPROVE ALL OF THEM IRREGARDLESS OF WHERE  
19 THEY FIT ON THE 85 TO 100 RANKING SCALE.

20 SO FROM MY POINT OF VIEW, THEN, I PUT THAT  
21 ASIDE AND LOOK AT WHAT OTHER ATTRIBUTES WE MIGHT  
22 CONSIDER IN TERMS OF LOOKING AT THESE APPLICATIONS.  
23 AND SO THIS CHART THAT'S BEEN PROVIDED TO US PRIOR  
24 TO THE MEETING AND WHICH REFERENCE HAS BEEN MADE TO  
25 A COUPLE OF TIMES FOR ME IS ILLUSTRATIVE OF

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1 SOMETHING THAT MIGHT WE WELL CONSIDER, AND THAT IS  
2 HOW WELL FUNDED ARE THESE PARTICULAR THERAPEUTIC  
3 AREAS ALREADY.

4 SO YOU WILL SEE A VAST DISPARITY FROM SOME  
5 DISEASE INDICATIONS THAT HAVE NO FUNDING. TAKE, FOR  
6 EXAMPLE, THE VERY FIRST ONE, DANON DISEASE. NOW, IT  
7 HAS A VERY HIGH SCIENTIFIC SCORE; BUT PUTTING THAT  
8 ASIDE, THERE ARE NO --

9 MR. TORRES: EXCUSE ME, STEVE. PLEASE PUT  
10 YOUR PHONES ON MUTE.

11 MR. JUELSGAARD: ARE WE GOOD TO GO NOW?  
12 I'LL CONTINUE. ANYWAY, THERE ARE A NUMBER OF THESE  
13 THAT HAVE EITHER NO CURRENT ACTIVITY GOING ON WITH  
14 RESPECT TO THIS DISEASE OR THERAPEUTIC AREA OR ONLY  
15 A MINIMAL AMOUNT OF ACTIVITY. AND ON THE OTHER  
16 HAND, THERE ARE SOME WHERE THERE'S A GREAT DEAL OF  
17 ACTIVITY THAT WE'VE ALREADY FUNDED. AND SO THERE'S,  
18 FOR ME, A BIT OF AN EQUITY ISSUE IN ALL OF THAT, AND  
19 THAT RELATES TO WHETHER OR NOT WE SHOULDN'T BE  
20 FUNDING THOSE HAVING SOME FUNDING IN THOSE AREAS  
21 THAT CURRENTLY WE HAVE NO PROJECTS BEING ADDRESSED.  
22 SO I THINK THAT'S AN IMPORTANT, AT LEAST FOR ME,  
23 CONSIDERATION THAT I WOULD PUT INTO THE MIX.

24 SO THAT STANDS SEPARATE AND APART FROM  
25 SCIENTIFIC MERIT. AGAIN, I GO BACK TO MY



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1 PROPOSITION THAT ONCE THEY'RE IN THAT TOP-TIER  
2 GROUP, THEY'RE ALL WORTHY FROM A SCIENTIFIC POINT OF  
3 VIEW. SO NOW IT GETS TO BE A QUESTION OF ARE THEY  
4 WORTHY FROM SOME OTHER POINT OF VIEW? AND SO, AT  
5 LEAST, AND I'M REPEATING MYSELF, BUT ONE VERY  
6 SIMPLISTIC WAY OF LOOKING AT THIS IS WHICH ARE THE  
7 AREAS THAT RECEIVE NONE OR MINIMAL FUNDING AT THIS  
8 POINT? AND SHOULDN'T WE BE DEDICATING RESOURCES TO  
9 THOSE AS OPPOSED TO SOME OF THESE WHICH HAVE AS MANY  
10 AS 35 PROJECTS, GENERALLY SPEAKING, INVOLVED AS, FOR  
11 EXAMPLE, IN THE CANCER AREA. SO, ANYWAY, THAT'S MY  
12 THOUGHTS.

13 DR. MARTIN: STEVE, I THINK THOSE ARE VERY  
14 VALID THOUGHTS, AND I WAS OBVIOUSLY NOT INVOLVED IN  
15 THE EARLIER PROCESS THAT CONSIDERED PROGRAMMATIC  
16 ISSUES. BUT I THINK THERE ARE TWO OTHERS THAT WE  
17 DID DISCUSS THAT WE CAN'T LOSE SIGHT OF. ONE WAS  
18 BROUGHT UP BY ART, AND THAT IS WE'RE SPENDING THE  
19 PUBLIC'S MONEY ON THIS. AND SO ONE CRITERION SHOULD  
20 BE WHAT IS THE IMPACT, SOCIETAL IMPACT, ON  
21 CALIFORNIANS, FOR EXAMPLE? AND THAT HAS SORT OF A  
22 SIZE ISSUE TO IT.

23 AND THEN THE OTHER ONE, WHICH I THINK ALSO  
24 NEEDS TO BE WEIGHED IN IS WHETHER A PROJECT IS  
25 INNOVATIVE ENOUGH SO IT'S GOING TO HAVE WIDESPREAD

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1 APPLICATIONS THAT, FOR EXAMPLE, WOULD GO BEYOND JUST  
2 THE NUMBER OF INDIVIDUALS OR PATIENTS AFFECTED WITH  
3 A SPECIFIC PROPOSAL AND/OR WHETHER THE APPLICANT HAS  
4 RECEIVED OR THE FIELD HAS RECEIVED PREVIOUS  
5 ACTIVITY.

6 SO IT'S A COMPLEX -- I CONSIDER IT TO BE  
7 AN IMPORTANT BUT COMPLEX PROBLEM WITH AT LEAST THREE  
8 TYPES OF CRITERIA THAT NEED TO BE CONSIDERED.

9 DR. JUELSGAARD: SO, DAVE, I APPRECIATE  
10 THAT. IN THE LANGUAGE IN THE TEXT OF PROPOSITION  
11 71, THERE'S A REFERENCE, AT LEAST IN A COUPLE OF  
12 DIFFERENT PLACES, TO THE SUPPORT FOR ORPHAN  
13 INDICATIONS. THIS ADDRESSES YOUR ISSUE OF IMPACT,  
14 RIGHT? SO YOU'RE SPEAKING OF DISEASE POPULATIONS  
15 WHICH ARE LARGE IN NUMBER AND LARGE IN ECONOMIC  
16 IMPACT. BUT SEPARATE AND APART FROM THAT ARE THE  
17 SO-CALLED ORPHAN INDICATIONS, THE ONES THAT REALLY  
18 DON'T GET MUCH ATTENTION AT ALL. THEY GET  
19 OVERLOOKED BECAUSE THERE ISN'T THE LEVEL, CERTAINLY,  
20 OF COMMERCIAL INTEREST OR PERHAPS EVEN RESEARCH  
21 INTEREST ASSOCIATED WITH THOSE.

22 SO I DO THINK WE NEED TO PUT THOSE ON THE  
23 SAME PLAYING FIELD WITH THE LARGE INDICATIONS, IF  
24 FOR NO OTHER REASON, BECAUSE THAT SPECIFICALLY IS  
25 CALLED OUT IN THE LANGUAGE OF PROP 71.

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1 DR. MARTIN: GOOD POINT. THANK YOU.

2 MR. SHEEHY: CHAIRMAN THOMAS.

3 CHAIRMAN THOMAS: ANOTHER SORT OF WAY OF  
4 LOOKING AT THIS WITH RESPECT TO THE PROJECTS THAT  
5 HAVE SIGNIFICANT AMOUNT OF FUNDING TO DATE OR A  
6 PARTICULAR INDICATION THAT DOES, ONE WAY OF LOOKING  
7 AT THOSE IS, YES, THAT IS A FACTUAL STATEMENT. BUT  
8 I ALWAYS SORT OF FALL BACK ON OUR CHILEAN MINER  
9 EXAMPLE WHERE THEY ENDED UP BORING THREE SEPARATE  
10 HOLES INTO THE EARTH IN THE HOPE THAT ONE OF THEM  
11 HIT THE MINERS, WHICH ULTIMATELY WAS THE CASE. AND  
12 THE ARGUMENT CAN BE MADE THAT IF YOU HAVE  
13 INDICATIONS THAT THE BOARD FEELS ARE A PRIORITY, THE  
14 FACT THAT WE HAVE GIVEN FUNDING TO IT DOES NOT  
15 NECESSARILY COUNT OUT GIVING ADDITIONAL FUNDING WITH  
16 DEVIATING STRATEGY THAT MIGHT GIVE A BETTER SHOT OF  
17 ULTIMATELY ACHIEVING THE GOAL OF GETTING A CURE FOR  
18 THAT PARTICULAR INDICATION.

19 SO I WOULD ASK THAT WE SORT OF KEEP THAT  
20 AS AN ADDITIONAL THING TO ANALYZE IN THE COURSE OF  
21 THIS DISCUSSION.

22 DR. HIGGINS: MAY I MAKE A COMMENT.

23 MR. SHEEHY: SURE.

24 DR. HIGGINS: JUST A QUICK COMMENT. THIS  
25 REALLY IS JUST AN EXTENSION OF WHAT DAVE SAID. IS

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1     THERE SOME CRITERIA THAT WE ALREADY JUDGE THESE  
2     APPLICATIONS ON THAT WOULD BE SORT OF THE CRITERION  
3     NO. 4 OR CRITERION NO. D OR WHATEVER? AFTER THE TOP  
4     THREE HAVE DEMONSTRATED SCIENTIFIC WORTHINESS, IS  
5     THERE SOME OTHER CRITERION THAT WE HAVE ALREADY  
6     RULED ON, IF YOU WILL, THAT WE COULD AUTOMATICALLY  
7     SORT OF FOLD INTO THAT PLACE? I DON'T KNOW IF I'M  
8     MAKING SENSE. WHETHER IT BE AN INDICATION OR  
9     WHETHER IT WAS A SUCCESS OR THE TEAM'S CONFIDENCE,  
10    THAT KIND OF THING. THAT'S MY QUESTION.

11           MR. SHEEHY: TO WHOM ARE YOU ADDRESSING  
12    THIS QUESTION?

13           DR. HIGGINS: TO THE GROUP, TO YOU, TO  
14    J.T. I GUESS WHAT I'M THINKING -- TRYING TO THINK  
15    IS IS THERE AN AUTOMATIC DEFAULT NEXT CRITERIA TO  
16    JUDGE GRANTS ON AFTER THEY'VE ALL PASSED THE 85  
17    MARK? WHAT STEVE SAID I THINK IS REALLY TRUE.  
18    ANYTHING SORT OF 86 THROUGH A HUNDRED IS KIND OF THE  
19    SAME. SO WE'RE LOOKING FOR SOME WAY TO DISTINGUISH  
20    THOSE. INSTEAD OF JUST SORT OF HAVING A RANDOM  
21    DISCUSSION ABOUT IT, IS THERE SOME WAY WE CAN HAVE  
22    AN AUTOMATIC DEFAULT NEXT CRITERIA TO SEPARATE  
23    THESE?

24           MR. SHEEHY: AT LEAST FOR ME PERSONALLY A  
25    COUPLE OF THINGS THAT ALWAYS IMPACT ME ARE HOW

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1 ALIGNED IS IT WITH THE ORIGINAL MISSION OF CIRM. SO  
2 AS I SAID, I HAVE A LOT OF TROUBLE WITH SMALL  
3 MOLECULES, WHICH THERE'S NO REAL IMPEDIMENT TO  
4 DEVELOPING. AND TO ME ALSO I THINK ANOTHER BIG  
5 CRITERIA FOR ME OR CRITERION IS I TAKE SERIOUSLY THE  
6 FACT THAT CIRM HAS ALREADY INVESTED IN THESE  
7 PROJECTS. AND SO IF WE DECIDE TO NOT CONTINUE TO  
8 INVEST, I PERSONALLY LIKE TO HAVE A STRONG REASON.  
9 AND THEN I DO LOOK AT INDICATION AND ALSO TO WHAT  
10 DEGREE YOU'RE CREATING INNOVATION WHERE WE CAN  
11 REALLY SHOW REAL LEADERSHIP.

12 SO THAT'S KIND OF -- BUT I DO THINK, AT  
13 LEAST FROM MY PERSPECTIVE, I THINK AT SOME POINT  
14 WE'LL HAVE TO LOOK AT THE APPLICATIONS AND ASK  
15 PEOPLE TO MAKE MOTIONS TO FUND. AND THEN IT WILL BE  
16 UP TO THE MOTION MAKER TO PRESENT AN ARGUMENT THAT  
17 MAY INCLUDE SOME, IF NOT ALL OF THE DIFFERENT  
18 CONSIDERATIONS THAT WE'VE DISCUSSED TODAY, AND WE  
19 HAVE TO MAKE A CHOICE. WE HAVE LIMITED RESOURCES.  
20 WE HAVE NOT GOTTEN TO A POINT WHERE WE HAVE  
21 IDENTIFIED SPECIFIC CRITERIA IN ORDER TO DO THAT  
22 CUT. AND I THINK, GIVEN THE RANGE OF ELEMENTS WE'VE  
23 DISCUSSED, I DON'T KNOW IF WE HAVE THE TIME OR  
24 ABILITY TO KIND OF COME UP WITH ONE OR TWO CRITERIA  
25 THAT WE CAN USE TODAY, IN MY OPINION, JUST TO GET AN

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1 INITIAL CUT.

2 DR. HIGGINS: THANKS FOR THAT, JEFF. I  
3 APPRECIATE THAT.

4 DR. STEWARD: COULD I GET IN THE CUE?

5 MR. SHEEHY: GO AHEAD.

6 DR. STEWARD: TWO THINGS. ONE IS THAT  
7 THERE IS AT LEAST AN OBJECTIVE CRITERION THAT WE  
8 COULD CONSIDER, AND I'M NOT SAYING IT'S NECESSARILY  
9 THE ONE THAT I WOULD LOOK TO IMMEDIATELY, BUT IT IS  
10 THE NUMBER OF REVIEWERS THAT RECOMMENDED AGAINST  
11 FUNDING. THAT INFORMATION IS AVAILABLE. AND SO  
12 SOME OF THESE HAVE ZERO RECOMMENDING AGAINST, SOME  
13 HAVE ONE, SOME HAVE TWO, AND ONE HAS FOUR.

14 NOW, THAT JUST IS SORT OF SAYING THAT,  
15 YEAH, THERE WERE SOME NUMBER OF PEOPLE IN THE ROOM,  
16 BUT STILL THE AVERAGE WAS ABOVE 85. BUT THAT IS AT  
17 LEAST AN OBJECTIVE CRITERION IF YOU WANT TO LOOK AT  
18 THAT.

19 WHERE I AM HAVING A PROBLEM, AND IN  
20 PRINCIPLE I AGREE WITH STEVE AND OTHERS, THAT IT IS  
21 REALLY THE JOB OF THE ICOC TO MAKE THESE KINDS OF  
22 DECISIONS BASED ON PROGRAMMATIC CONSIDERATIONS.  
23 HOWEVER, I'M A LITTLE UNCERTAIN ABOUT HOW TO  
24 PROCEED. SO WE HAVE A LIMITED AMOUNT OF FUNDING.  
25 SO IF WE RECOMMEND PROJECT X TO BE MOVED INTO TIER

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1 I, WHERE DO WE PUT IT? BECAUSE WE CAN'T FUND ALL  
2 THAT ARE THERE ABOVE THE FUNDING LINE RIGHT NOW  
3 WITHOUT BUMPING ONE OTHER DOWN. SO I'M NOT EVEN  
4 SURE HOW TO MAKE THE MOTION. WE WOULD ALMOST HAVE  
5 TO MAKE IT I WOULD LIKE TO PUT IT IN THE FOLLOWING  
6 ORDER. SO I JUST THROW THAT OUT THERE AS A  
7 PROCEDURAL ISSUE TO THINK ABOUT AS WE'RE DISCUSSING  
8 THIS. THANK YOU.

9 MR. SHEEHY: THANK YOU. DO WE HAVE OTHER  
10 CONSIDERATIONS OR COMMENTS FROM OTHER MEMBERS?

11 DR. JUELSGAARD: SO I WANT TO RESPOND TO  
12 SOMETHING THAT J.T. SAID ABOUT THE CHILEAN MINER WAY  
13 OF LOOKING AT THE WORLD. IF YOU TAKE A DISEASE AREA  
14 LIKE CANCER, FOR EXAMPLE, CANCER AND CARDIOVASCULAR  
15 DISEASE AND HEART ISSUES ARE THE TWO MOST FUNDED  
16 THERAPEUTIC AREAS OR DISEASE AREAS GLOBALLY TODAY.  
17 IT'S NOT JUST CIRM THAT'S PROVIDING FUNDING FOR  
18 PROGRAMS, BUT THERE'S A VAST EFFORT THAT'S BEING  
19 UNDERTAKEN BY MANY, MANY INSTITUTIONS BOTH  
20 FOR-PROFIT AND NON-FOR-PROFIT. AND SO ONE MORE MINE  
21 SHAFT WHERE WE'RE LITTERED WITH MILLIONS OF MINE  
22 SHAFTS I DON'T THINK IS NECESSARILY A HELPFUL WAY OF  
23 LOOKING AT IT. I'M MORE WORRIED ABOUT THE PLACES  
24 WHERE THE CHILEAN MINERS ARE TRAPPED AND NOBODY IS  
25 DRILLING A HOLE TO GET TO THEM, TO USE THAT ANALOGY.

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1 MR. SHEEHY: OKAY. OTHER MEMBERS HAVE  
2 POINTS THEY'D LIKE TO MAKE OR SHOULD WE MOVE AHEAD?  
3 OKAY.

4 SO I THINK AT THIS POINT I THINK WE'RE  
5 LOOKING AT ONE OF TWO MOTIONS. EITHER A MOTION TO  
6 FUND AN APPLICATION OR A MOTION TO NOT FUND AN  
7 APPLICATION. I THINK WE CAN -- IS THERE A  
8 PREFERENCE FOR STARTING FROM THE TOP, OR DO WE WANT  
9 TO GO AT IT JUST BASED ON WHAT FOLKS WOULD LIKE TO  
10 DO?

11 DR. JUELSGAARD: YES. SO I KNOW THIS IS A  
12 LITTLE COMPLICATED, JEFF, BUT THERE IS ONE OTHER WAY  
13 OF LOOKING AT THIS. AND SO REMEMBER WHAT WE'RE  
14 LIMITED BY IS BY \$10 MILLION, BUT THAT LIMITATION IS  
15 FOR THIS FISCAL YEAR ONLY, WHICH ENDS, I THINK, ON  
16 DECEMBER 31ST FROM A FUNDING OF GRANTS POINT OF  
17 VIEW. SO ONE ALTERNATIVE IS TO, IN ESSENCE, APPROVE  
18 ALL OF THESE IN WHATEVER ORDER WE WANTED TO APPROVE  
19 THEM, BUT SIMPLY PROVIDE FUNDING FOR THOSE THAT WE  
20 SO DEEM WORTHY RIGHT NOW. AND THEN IN THE NEXT  
21 BUDGET CYCLE, WHICH COMES UP AT THE BEGINNING OF  
22 NEXT YEAR AND WHEN WE APPROVE FUNDING FOR ANOTHER  
23 GROUP OF QUEST APPLICATIONS, WE ALREADY HAVE A GROUP  
24 OF AUTOMATICALLY APPROVED APPLICATIONS THAT WOULD  
25 RECEIVE THAT FUNDING.



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1 SO, IN OTHER WORDS, THERE WOULD BE ONE  
2 GROUP THAT RECEIVED FUNDING NOW, AND THEN THE OTHER  
3 GROUP WOULD RECEIVE FUNDING, LET'S SAY, FIVE MONTHS  
4 FROM NOW, WHICH IS ROUGHLY IN THE BEGINNING OF WHEN  
5 NEXT YEAR COMMENCES. SO THAT WE SAY, OKAY, ALL YOU  
6 THESE ARE WORTHY, AND WE SHOULD AGREE TO FUND THEM.  
7 WE'RE JUST GOING TO DO THIS IN TWO TRANCHES, THE  
8 MONEY WE HAVE NOW AND THEN THE MONEY WE'LL MAKE  
9 AVAILABLE NEXT YEAR.

10 SO PERHAPS, OS, THIS CIRCUMVENTS THE WHOLE  
11 ISSUE OF TRYING TO FIGURE OUT WHICH IS THE WORTHIEST  
12 RIGHT NOW, WHETHER IT'S ALL BASED ON SCIENCE OR  
13 WHETHER IT'S BASED ON A NUMBER OF PROJECTS ONGOING  
14 OR WHATEVER WE WANTED TO USE. SO I JUST THROW THAT  
15 OUT THERE AS AN ALTERNATIVE. I REALIZE THERE MAY BE  
16 SOME ISSUES WITH THAT THAT WE OUGHT TO TALK ABOUT,  
17 BUT A DIFFERENT WAY OF LOOKING AT IT.

18 DR. STEWARD: IF I COULD, I THINK THERE  
19 ARE VERY IMPORTANT ISSUES AND PROBLEMS WITH THAT  
20 APPROACH. THE FIRST AND MOST OBVIOUS IS THAT IT  
21 KIND OF ROLLS INTO NEXT YEAR THIS SET OF GRANTS  
22 WHICH BY NEXT YEAR MAY NOT BE THE TOP ONES THAT WE  
23 WOULD WANT TO FUND IF WE WERE RECOMPETING THE WHOLE  
24 THING.

25 THE SECOND IS THAT IT IMPACTS IN A VERY

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1 CRITICAL WAY ON NEXT YEAR'S BUDGET CONSIDERATION.  
2 SO I THINK THAT'S A MUCH -- THAT SECOND POINT BRINGS  
3 US INTO A MUCH BROADER CONVERSATION. BUT JUST ON  
4 THE BASIS OF THE FIRST, I THINK THAT THIS IS  
5 PROBABLY NOT THE WAY WE WOULD WANT TO DO THIS IF WE  
6 ARE REALLY LOOKING TO FUND THE VERY BEST THINGS THAT  
7 WE HAVE AVAILABLE AT THE TIME THAT WE HAVE THE FUNDS  
8 AVAILABLE. THANK YOU.

9 DR. JUELSGAARD: OS, THAT SORT OF BEGS THE  
10 QUESTION OF WHAT ARE THE BEST TO BE APPROVED. SO MY  
11 ARGUMENT, IT'S NOT BASED ON THE SCIENTIFIC SCORE,  
12 BUT IT'S BASED ON SOME OTHER ANALYSIS. SO I'M NOT  
13 SURE HOW YOU CAN FIGURE OUT WHAT'S BEST AND WHAT'S  
14 NOT BEST. WE'LL RUN INTO THE SAME PROBLEM NEXT TIME  
15 IF WE PROCEED WITH ONLY FUNDING 10 MILLION AND  
16 EVERYBODY COME BACK AROUND AGAIN. WE'RE GOING TO  
17 GET TO PICKING AND CHOOSING IN SOME FASHION  
18 ULTIMATELY.

19 DR. STEWARD: NO. I AGREE. I'M JUST  
20 SAYING THAT, YES, I THINK WE NEED TO FORCE OURSELVES  
21 TO HAVE THE DISCIPLINE TO DO THAT NOW. IT WOULD BE  
22 FINE FOR THESE PROJECTS TO COME BACK IN NEXT YEAR  
23 AND RECOMPETE, AND THAT WOULD BE GREAT, AND WE'RE  
24 GOING TO HAVE TO DO IT AGAIN NEXT YEAR. BUT THAT IS  
25 ALL PART OF THE PROCESS. THANK YOU.

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1                   CHAIRMAN THOMAS:    COUPLE POINTS ON THAT.  
2                   FYI, THE NEXT ROUND OF QUEST AWARDS, I BELIEVE, ARE  
3                   TARGETED TO BE CONSIDERED AT THE SAME TIME OF THE  
4                   YEAR NEXT YEAR AS THIS YEAR.   AM I CORRECT, DR.  
5                   SAMBRANO?   THAT ROUND WOULDN'T BE UNTIL JUNE, NO. 1.

6                   NO. 2, THIS NOTION OF PROVIDING FUNDING,  
7                   WE GET INTO THE NEXT BUDGET CYCLE.   MR. TOCHER, IS  
8                   THAT REMINISCENT OF IN THE OLD DAYS THERE WAS A  
9                   DESIGNATION FOR PROVIDING -- APPROVING PROJECTS FOR  
10                  WHICH FUNDS ARE AVAILABLE SO THAT PRESUMABLY IF  
11                  FUNDS BECAME AVAILABLE SUBSEQUENT TO THAT PARTICULAR  
12                  DATE, THEY WOULD BE FUNDED?   OR WAS THAT NOT THE  
13                  PURPOSE OF THAT DESIGNATION?   WE HAVEN'T HAD THAT  
14                  ONE FOR MANY YEARS.

15                  MR. TOCHER:   RIGHT.   VERY EARLY ON THERE  
16                  WAS A CATEGORY IF FUNDS ARE AVAILABLE, BUT NEITHER  
17                  OF THOSE DECISIONS WERE ACTUALLY MADE.   THE  
18                  DETERMINATION THAT FUNDS WERE OR WERE NOT AVAILABLE  
19                  WERE ACTUALLY MADE AT THE BOARD MEETING WHEN ALL OF  
20                  THE OTHER APPLICATIONS WERE DISCUSSED.   SO THOSE  
21                  ROUNDS WERE, IN FACT, CLOSED OUT ALL AT THE SAME  
22                  TIME ALONG WITH THOSE APPLICATIONS THAT WERE  
23                  RECOMMENDED FOR FUNDING.   THAT WAS WHEN WE HAD MORE  
24                  MONEY AND THE BOARD FELT MORE COMFORTABLE WITH  
25                  AUGMENTING ITS BUDGET SORT OF ON THE FLY.

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1 CHAIRMAN THOMAS: THANK YOU FOR THE  
2 CLARIFICATION.

3 MS. WINOKUR: THIS IS DIANE. I WONDER IF  
4 WE'VE EVER MADE SOME CONSIDERATION TO THE  
5 INSTITUTION THAT'S REPRESENTED BY THE FUNDS WE  
6 GRANT. THERE ARE SOME INSTITUTIONS THAT WE HAVE  
7 GIVEN A LOT OF MONEY TO BY FUNDING PROJECTS THAT  
8 CAME FROM THEM AND SOME INSTITUTIONS WHERE WE'VE  
9 NEVER GIVEN ANY MONEY THAT HAVE APPLIED THIS TIME.  
10 I'M JUST ASKING WHETHER WE'VE EVER DONE THAT.

11 MR. SHEEHY: WE HAVEN'T BECAUSE WE'RE  
12 UNABLE TO IDENTIFY THE PRIMARY INVESTIGATOR AND THE  
13 INSTITUTION PRIOR TO THE AWARD PUBLICLY. WE'RE  
14 UNABLE TO PUBLICLY IDENTIFY. OUR PROCESS HAS NOT  
15 PUBLICLY IDENTIFIED EITHER THE INSTITUTION OR THE  
16 PRIMARY INVESTIGATOR PRIOR TO AWARD. THAT'S BEEN  
17 THE PRACTICE. NOW, SOMETIMES THEY SELF-DISCLOSE  
18 THROUGH APPEALS OR LETTERS OR OTHER MANNER OF  
19 CONTACT, BUT IT'S ALWAYS BEEN OUR PROCESS NOT TO DO  
20 SO.

21 SO ARE THERE OTHER THOUGHTS,  
22 CONSIDERATIONS? ARE THERE ANY MOTIONS? I THINK  
23 WE'LL BE HERE AWHILE. I THINK WE PROBABLY SHOULD  
24 CLOSE OUT THAT DISCUSSION. AND I THINK -- STEVE.

25 DR. JUELSGAARD: SO I'D ACTUALLY LIKE JUST

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1 TO -- I THINK THIS WILL HELP US REACH SOME  
2 RESOLUTION. SO I'M GOING TO MOVE THAT WE APPROVE A  
3 SINGLE APPLICATION AT THIS POINT, WHICH IS 11070,  
4 WHICH IS ENTITLED "DRUG DEVELOPMENT FOR AUTISM  
5 SPECTRUM DISORDER USING HUMAN PATIENT IPSC'S." AND  
6 IT'S A PROJECT FOR WHICH WE ONLY HAVE ONE  
7 APPLICATION THAT WE'VE APPROVED HISTORICALLY. SO WE  
8 HAVE VERY LITTLE ACTIVITY IN THAT AREA FOR A DISEASE  
9 OR CONDITION THAT'S SEEMINGLY VERY WIDESPREAD IN THE  
10 POPULATION THESE DAYS. IT AFFECTS YOUNG PEOPLE. SO  
11 I MOVE THAT WE APPROVE THAT APPLICATION. IT'S NOT  
12 AMONG THE TOP SEVEN FROM A SCIENTIFIC RANKING POINT  
13 OF VIEW.

14 DR. STEWARD: STEVE, THIS IS OS, AND IF I  
15 COULD JUST ASK A QUESTION. ACTUALLY I'M GOING TO GO  
16 AHEAD AND SECOND THIS. AND THE REASON IS THAT WE  
17 HAVE TRADITIONALLY HAD A VERY SERIOUS  
18 UNDERREPRESENTATION OF DISEASES AFFECTING CHILDHOOD  
19 MENTAL HEALTH. BUT I DO WANT TO ASK YOU A QUESTION.  
20 SO WHERE DO YOU PUT IT? ARE YOU SUGGESTING THAT  
21 THIS REPLACE WHAT IS CURRENTLY RANKED NO. 7? THANK  
22 YOU.

23 DR. JUELSGAARD: NO, IT DOESN'T REPLACE  
24 ANYTHING. OS, MY PREMISE IS THAT ONCE YOU'RE IN  
25 THIS GREEN CHART THAT WE HAVE UP IN FRONT OF US HERE

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1 IN OAKLAND, ONCE YOU'RE THERE, ONCE YOU'VE GOT A  
2 SCORE OF 85 AND ABOVE, YOU'RE IN WHAT I CALL THE  
3 WORTHY CATEGORY. AND I DON'T PLACE A LOT OF  
4 DISTINGUISHMENT -- A LOT OF DISTINCTION AMONGST  
5 THESE. I DO REFLECT UPON YOUR POINT AS FAR AS THE  
6 NUMBER OF NO VOTES GO, AND I SEE THAT THE VERY LAST  
7 TWO ON THIS GREEN CHART, ONE HAS, I THINK, FOUR NO  
8 VOTES AND THE OTHER HAS FIVE. I GIVE SOME CREDENCE  
9 TO THAT. BUT APART FROM THAT, FOR ME, THE OTHER 13  
10 ARE ON THE SAME PLAYING FIELD. I'M NOT REPLACING  
11 ONE FOR ANOTHER.

12 DR. STEWARD: STEVE, I'M SORRY. I THINK  
13 YOU HAVE TO BECAUSE OTHERWISE WE GO OVER THE BUDGET.  
14 AND WE CAN'T -- IF I UNDERSTAND IT, AGAIN, SOMEBODY  
15 CORRECT ME IF I'M WRONG, BUT WE CAN'T GO OVER THE  
16 BUDGET UNLESS THE BOARD VOTES TO EXCEED THAT BUDGET.  
17 AND THERE'S A PROBLEM DOING THAT, AS WE KNOW, WHEN  
18 CONSIDERING PARTICULAR APPLICATIONS. SO THIS WOULD  
19 HAVE TO BE EITHER A REPLACEMENT OR YOU WOULD HAVE TO  
20 CUT THE FUNDING FOR SOMETHING ABOVE THAT BUDGET  
21 LINE. THANK YOU.

22 DR. JUELGAARD: NO, OS. WHAT I'M  
23 PROPOSING IS WE DO THIS ON A ONE BY ONE BY ONE  
24 BASIS, NOT ON A GROUP BASIS. I'M NOT BUYING THE  
25 GROUP NOTION. WE DO THAT WHEN WE HAVE ENOUGH MONEY

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1 FOR EVERYBODY; BUT WHEN WE DON'T, THEN IT'S GOING TO  
2 BE WHICH ARE THE MOST WORTHY. IT'S WHATEVER THEY  
3 ARE. SO I'M STARTING WITH THIS ONE. AND MY  
4 SUGGESTION IS WE GO TO THE NEXT ONE THAT SOMEBODY  
5 FEELS IS WORTHY OF BEING FUNDED UNTIL WE HIT THE \$10  
6 MILLION MARK.

7 DR. STEWARD: I'M SORRY THEN. THIS MAY  
8 NOT BE LEGAL. I'M GOING TO ASK SCOTT. I'M GOING TO  
9 WITHDRAW MY MOTION IN THAT CASE, MY SECOND OF THAT  
10 MOTION. IS THAT -- MAY I DO THAT?

11 MR. TOCHER: YES.

12 MR. SHEEHY: DO WE HAVE AN ADDITIONAL  
13 SECOND? NOT HEARING A SECOND, CAN I GET ANOTHER  
14 MOTION?

15 DR. SAMBRANO: I JUST WANT TO MAKE A  
16 CLARIFICATION ON THE DOCUMENT THAT WAS PROVIDED JUST  
17 SO YOU KNOW. THE NUMBER OF AWARDS THAT ARE LISTED  
18 REFLECT ACTIVE AWARDS. SO IN MANY CASES THERE MAY  
19 HAVE BEEN ADDITIONAL AWARDS THAT, AT LEAST IN CIRM'S  
20 LIFETIME, MAY HAVE BEEN MADE TO SOME OF THESE  
21 INDICATIONS. SO WITH THE AUTISM, FOR EXAMPLE, WE  
22 HAVE IN THE PAST MADE OTHER AWARDS IN THAT ARENA.  
23 WE JUST HAPPEN TO HAVE ONE THAT WE CALL THE ANGELMAN  
24 SYNDROME THAT FALLS INTO THE AUTISM SPECTRUM  
25 DISORDERS GROUP.

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1 MR. SHEEHY: DO WE HAVE A MOTION? I'M  
2 HAPPY TO MAKE A MOTION IF NO ONE ELSE WANTS TO.

3 DR. STEWARD: LET ME TRY ONE HERE. I  
4 WOULD LIKE TO MOVE, AND I THINK WE CAN DO THIS AS A  
5 GROUP, THAT ALL OF THE APPLICATIONS THAT HAVE NO --  
6 IN WHICH NONE OF THE REVIEWERS RECOMMENDED AGAINST  
7 FUNDING, AND THERE ARE FOUR OF THOSE, THAT WE  
8 APPROVE THOSE FOUR. THAT'S NOS. 1, 2, AND 3 ON THE  
9 LIST AND NO. 5. THANK YOU.

10 DR. HIGGINS: I WOULD SECOND THAT MOTION.

11 MR. SHEEHY: WE HAVE A SECOND FOR THAT  
12 MOTION. DO WE HAVE DISCUSSION? AND ALSO SOMEBODY  
13 IS ADDING, I HOPE.

14 DR. SAMBRANO: JUST SO YOU KNOW, ON THIS  
15 SPREADSHEET, AND IF YOU CAN SEE IT ON WEBEX, IT WILL  
16 COUNT HOW MUCH IN TERMS OF FUNDS ARE AVAILABLE AFTER  
17 YOU HAVE APPROVED EACH OF THE APPLICATIONS. SO WE  
18 WILL TRACK THAT AMOUNT.

19 MR. SHEEHY: SO WE HAVE A MOTION AND A  
20 SECOND. IS THERE DISCUSSION?

21 MR. TORRES: WHICH IS THE PROJECT AGAIN?

22 MR. SHEEHY: SO THE PROJECTS ARE 11131  
23 THAT HAS A BUDGET OF 1.4 THAT IS FOR DANON DISEASE.  
24 IT'S CELL THERAPY. THEN IT'S 11157 AND IT'S 1.4  
25 MILLION, AND IT'S FOR THE UNIVERSAL THERAPY FOR



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1 CANCER. IT'S A CELL THERAPY. APPLICATION 1 AND 2.  
2 AND THEN APPLICATION 3, WHICH IS 11036, WHICH IS THE  
3 THIRD APPLICATION, IT IS FOR PEDIATRIC GLIOBLASTOMA,  
4 I BELIEVE. IS THAT CORRECT?

5 DR. SAMBRANO: YES.

6 MR. SHEEHY: THE FOURTH IS 11175, WHICH  
7 IS, I THINK, FOR TYPE 1 DIABETES, I BELIEVE.

8 SO IS THERE FURTHER DISCUSSION?

9 MR. TORRES: AND THE TOTAL OF THOSE  
10 RECOMMENDATIONS IS?

11 DR. SAMBRANO: SO THAT WOULD TOTAL TO 5.3  
12 MILLION.

13 MR. SHEEHY: AND WE HAVE ANOTHER 4.7  
14 AVAILABLE. IS THERE DISCUSSION? PEOPLE WANT TO  
15 DISCUSS? DO WE WANT -- WE NEED TO OPEN THIS UP FOR  
16 PUBLIC COMMENT. SO ANYWHERE. YOU CAN START AT THE  
17 SATELLITES IF THERE'S SOMEONE AT ONE OF THE  
18 SATELLITES WHO WOULD LIKE TO MAKE A PUBLIC COMMENT.

19 DR. CHIU: PUBLIC COMMENT FROM CITY OF  
20 HOPE.

21 MR. SHEEHY: YES.

22 DR. CHIU: THIS IS ARLENE CHIU FROM CITY  
23 OF HOPE. I WOULD APPRECIATE UNDERSTANDING THE  
24 RATIONALE FOR THESE FIVE BEING PROPOSED. I GUESS I  
25 MISSED IT BECAUSE OF THE DISTANCE AND ALL. JUST A

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1 CLARIFICATION. YOU ARE RECOMMENDING FUNDING NOW FOR  
2 THE FIRST, SECOND, AND THIRD, AND THEN THE FIFTH AND  
3 THE SEVENTH. AM I FOLLOWING YOU CORRECTLY?

4 MR. SHEEHY: FIRST, SECOND, AND THIRD AND  
5 THE SEVENTH.

6 DR. CHIU: DISCUSSION ON ANY OF THESE  
7 PROPOSALS, OR IS THERE A RATIONALE FOR SELECTING  
8 THESE? THANK YOU.

9 MR. SHEEHY: DR. CHIU, WE'RE RECOMMENDING  
10 THE FIRST, SECOND, AND THIRD, AND SEVENTH RANKED.  
11 AND THE RATIONALE IS THAT THEY RECEIVED UNANIMOUS  
12 SUPPORT FROM THE MEMBERS OF THE GRANTS WORKING  
13 GROUP.

14 DR. STEWARD: I'M SORRY. I MAY BE  
15 MISREADING. BUT WHAT I WAS -- SO THE FIRST THREE,  
16 YES; BUT THE FOURTH ON MY LIST WAS 11175.

17 MR. SHEEHY: YEAH.

18 DR. STEWARD: IS THAT NO. 7? I MUST BE  
19 LOOKING AT A DIFFERENT RANKING LIST. YOU'RE SCORING  
20 BY MEDIAN, NOT MEAN. WHICH ONE ARE WE USING FOR  
21 THIS RANKING?

22 DR. SAMBRANO: WE'RE USING THE MEDIAN AS  
23 THE METHOD FOR RANKING. SO THE FIRST THREE END UP  
24 BEING UNANIMOUS, AND THEN THE SEVENTH IN THAT RANK  
25 ORDER ENDS UP BEING UNANIMOUS.

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1 DR. STEWARD: THE MEAN RANKING, IF YOU  
2 WANTED TO USE THE MEAN, 11175 WOULD BE, WELL, ONE OF  
3 THE TOP FIVE.

4 DR. SAMBRANO: RIGHT. SO THE WAY THESE  
5 ARE RANKED, THEY'RE RANKED FIRST BY MEDIAN AND THEN  
6 ANYTHING THAT TIES IN MEDIAN IS THEN RANKED BY MEAN.

7 MR. TORRES: MR. CHAIRMAN.

8 MR. SHEEHY: YES.

9 MR. TORRES: ON THE ITEM -- GILBERT, ON  
10 THE ITEM 11165 DEALING WITH DEMENTIA, IS THAT RANKED  
11 LAST? IS THAT WHAT YOU'RE SAYING?

12 DR. SAMBRANO: YES.

13 MR. TORRES: AND WHAT WAS THE RATIONALE  
14 FOR RANKING IT LAST?

15 DR. SAMBRANO: THE MEDIAN SCORE.

16 MR. TORRES: THE MEDIAN SCORE. THEREFORE,  
17 GOING ALONG WITH STEVE'S DISCUSSION ON DEVELOPING AN  
18 AWARD FOR AUTISM BECAUSE WE DON'T HAVE MUCH IN THAT  
19 AREA, IF ANY, AND DEMENTIA WE HAVE NOTHING, DO WE  
20 SEE ANYTHING IN THE FUTURE ON DEMENTIA BECAUSE THAT  
21 IS THE HIGHEST COST ITEM FOR HEALTHCARE IN  
22 CALIFORNIA?

23 DR. SAMBRANO: I'M NOT SURE I UNDERSTAND  
24 THE QUESTION YOU'RE ASKING ME.

25 MR. TORRES: THE QUESTION I'M ASKING IS DO

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1 YOU KNOW OF ANYTHING IN THE PIPELINE THAT WE WOULD  
2 CONSIDER LATER TO ADDRESS DEMENTIA?

3 DR. SAMBRANO: NO. WE DON'T HAVE ANYTHING  
4 CURRENTLY IN THE PIPELINE THAT WOULD BE DEVELOPING A  
5 PROJECT FOR AT LEAST THIS PARTICULAR FRONTOTEMPORAL  
6 DEMENTIA.

7 MR. TORRES: NOTHING THAT YOU FORESEE FROM  
8 POTENTIAL FUTURE APPLICATIONS?

9 DR. SAMBRANO: NO.

10 MR. SHEEHY: I WOULD BE HAPPY TO RECOGNIZE  
11 YOU, SENATOR TORRES, AFTER THIS MOTION IF YOU'D LIKE  
12 TO MAKE A MOTION FOR THAT APPLICATION.

13 MR. TORRES: THANK YOU, MR. CHAIRMAN.

14 MR. SHEEHY: MAYBE WE CAN WORK OUR WAY  
15 THROUGH THIS MOTION. DO WE HAVE ANY OTHER PUBLIC  
16 COMMENT?

17 DR. CHIU: I'M SORRY TO BUTT IN. THIS IS  
18 ARLENE CHIU AGAIN.

19 MR. SHEEHY: YOU NEVER BUTT IN. PLEASE.

20 DR. CHIU: I WAS JUST CURIOUS THAT THE  
21 REVIEWERS, THE GWG, HAVE GIVEN GREAT SERVICE IN  
22 IDENTIFYING AND SCORING AND IN THE YES/NO. AS ONE  
23 OF THE BOARD MEMBERS HAD MENTIONED, ALL THE  
24 PROPOSALS IN GREEN HAVE MET THE BASE CRITERION. AND  
25 I WAS UNDER THE IMPRESSION THAT THE BOARD WILL NOW

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1 LOOK WITH MANY OF THE CRITERIA THAT YOU HAVE  
2 IDENTIFIED IN THE EARLIER DISCUSSION. GOING BACK TO  
3 SELECTING THESE FOUR MEANS PICKING THE ONES WHERE,  
4 AGAIN, THE GWG HAS NOT VOTED NO, WHICH MEANS, AGAIN,  
5 DEFERRING TO GWG. I JUST WANTED TO POINT OUT THAT I  
6 WONDERED IF ANY OF THESE ALSO MEET YOUR OTHER  
7 CRITERIA. JUST A THOUGHT. THANK YOU.

8 DR. STEWARD: IF I COULD JUST COMMENT IN  
9 ANSWER TO YOUR QUESTION, ARLENE. WHAT I'M REALLY  
10 TRYING TO DO IS SORT OF GET US STARTED. AND I  
11 TOTALLY ACCEPT THAT WE WILL NEED TO DISCUSS THE  
12 OTHER CRITERIA IF THIS IS APPROVED, FILL IN THE LAST  
13 THREE APPLICATIONS TO BE FUNDED. THAT WAS REALLY  
14 THE REASON FOR DOING IT EXACTLY IN THE WAY THAT I'M  
15 DOING IT, THE WAY THAT I FORMULATED THIS MOTION.  
16 THANK YOU.

17 MR. SHEEHY: IS THERE FURTHER DISCUSSION  
18 OR PUBLIC COMMENT AT ANY SITE? COULD WE CALL THE  
19 ROLL THEN.

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

22 DR. HIGGINS: YES.

23 MS. BONNEVILLE: STEVE JUELSGAARD.

24 MR. JUELSGAARD: YES.

25 MS. BONNEVILLE: DAVE MARTIN.

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1 DR. MARTIN: AYE.  
2 MS. BONNEVILLE: LAUREN MILLER.  
3 MS. MILLER: YES.  
4 MS. BONNEVILLE: ADRIANA PADILLA.  
5 DR. PADILLA: YES.  
6 MS. BONNEVILLE: FRANCISCO PRIETO.  
7 DR. PRIETO: AYE.  
8 MS. BONNEVILLE: ROBERT QUINT.  
9 DR. QUINT: ABSTAIN.  
10 MS. BONNEVILLE: AL ROWLETT.  
11 MR. ROWLETT: YES.  
12 MS. BONNEVILLE: JEFF SHEEHY.  
13 MR. SHEEHY: YES.  
14 MS. BONNEVILLE: OS STEWARD.  
15 DR. STEWARD: YES.  
16 MS. BONNEVILLE: JONATHAN THOMAS.  
17 CHAIRMAN THOMAS: YES.  
18 MS. BONNEVILLE: ART TORRES.  
19 MR. TORRES: AYE.  
20 MS. BONNEVILLE: DIANE WINOKUR.  
21 MS. WINOKUR: YES.  
22 THE REPORTER: THIS IS BETH. I DIDN'T  
23 HEAR AN ANSWER FROM DR. DULIEGE.  
24 MS. BONNEVILLE: SHE HAS DROPPED OFF THE  
25 LINE.

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1 MR. SHEEHY: SENATOR TORRES, DID YOU HAVE  
2 A MOTION?

3 MR. TORRES: NO. I THINK, GIVEN THE  
4 EXPLANATION THAT GILBERT MADE ON THE VIABILITY OF  
5 THAT DEMENTIA PROJECT, I THINK I'M JUST GOING TO  
6 HAVE TO WAIT TILL SOMETHING COMES THROUGH OUR  
7 PROCESS BECAUSE I DO THINK IT'S AN ISSUE THAT WE  
8 NEED TO ADDRESS AS A STATE AGENCY AND THE IMPACT  
9 THAT IT HAS ON THE COST OF HEALTHCARE AND CAREGIVING  
10 IN CALIFORNIA. THANK YOU.

11 MR. SHEEHY: I THOUGHT THAT WAS A GOOD  
12 PROJECT. I WAS HOPING YOU'D MAKE A MOTION.

13 MR. TORRES: BUT IT CAME IN LAST. IS IT  
14 VIABLE SCIENTIFICALLY?

15 MR. SHEEHY: WELL, IT WAS IN THE FUNDABLE  
16 CATEGORY, SENATOR TORRES. I WOULD JUST NOTE THAT  
17 IT'S ONE OF THE MORE INNOVATIVE ONES AND THAT IT  
18 INVOLVES HUMAN EMBRYONIC STEM CELL-DERIVED -- NO,  
19 IPS, INDUCED PLURIPOTENT STEM CELL-DERIVED  
20 MICROGLIA.

21 MR. TORRES: I'M SORRY. THAT'S THE  
22 PROBLEM WITH BEING ON A CALL. YOU DON'T GET ALL THE  
23 INFORMATION IN FRONT OF YOU. SO I WOULD MOVE TO  
24 ACCEPT THAT PROJECT ON DEMENTIA.

25 MR. SHEEHY: YOU WANT A MOTION TO APPROVE

1 IT?

2 MR. TORRES: YES, SIR.

3 MR. SHEEHY: I WOULD BE HAPPY TO SECOND  
4 THAT MOTION.

5 IF PEOPLE ARE WONDERING, YOUR CRITERIA, I  
6 CERTAINLY AGREE WE HAVEN'T FUNDED ANYTHING IN THAT  
7 CATEGORY; BUT ALSO FOR ME IT IS HIGHLY INNOVATIVE  
8 AND VERY WELL ALIGNED WITH CIRM'S MISSION IN THAT IT  
9 REALLY IS AT THE CUTTING-EDGE OF BOTH, AT LEAST WHAT  
10 I UNDERSTAND THE ROLE OF MICROGLIA IN  
11 NEURODEGENERATIVE DISEASES IS STILL UNDEREXPLORED, I  
12 THINK. I SEE IT IN HIV, THAT WE REALLY DON'T KNOW  
13 WHAT'S HAPPENING WITH IMMUNE CELLS IN THE BRAIN AND  
14 WHAT THEIR ROLE IS. AND I WOULD NOTE TOO THAT IN  
15 HIV DEMENTIA, IT'S AN ISSUE THAT WE SEE, HAS ALWAYS  
16 BEEN AN ISSUE IN HIV, AND CERTAINLY AS PEOPLE AGE,  
17 IT'S AN ISSUE THAT GROWS.

18 IT GOT -- ONE WOULD EXPECT SOMETHING  
19 THAT'S HIGHLY INNOVATIVE TO PERHAPS NOT SCORE AS  
20 WELL AS MORE, LET'S SAY, PROJECTS WITH MORE GENERIC  
21 PATHWAYS. WE OBVIOUSLY DON'T HAVE AN IPSC APPROVED  
22 THERAPY. SO TO A LARGE DEGREE PEOPLE ARE KIND OF  
23 HUNTING TO FIGURE OUT HOW TO GET SOMETHING ACROSS  
24 THE FINISH LINE.

25 SO TO ME IT'S A VERY INNOVATIVE PROJECT.



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1 IT ALIGNS TOTALLY WITH THE CORE MISSION OF CIRM IN  
2 THAT IT INVOLVES PLURIPOTENT CELLS.

3 MR. TORRES: IT'S STARTING TO IMMERGE.  
4 I'M SPEAKING WITH MY OTHER HAT NOW AS A BOARD MEMBER  
5 OF COVER CALIFORNIA, WHICH OVERSEES OBAMACARE IN  
6 THIS STATE, THAT DEMENTIA IS COMING UP IN OUR  
7 NEGOTIATIONS WITH THIRD-PARTY PAYERS AS A REAL COST  
8 FACTOR. AND IF WE COULD POINT THAT WE'RE DOING  
9 SOMETHING, AT LEAST ATTEMPTING TO DO SOMETHING ABOUT  
10 THIS DISEASE, THEN I THINK THAT WOULD BE LOOKED ON  
11 FAVORABLY BY OUR POPULATION.

12 MR. TOCHER: SORRY TO INTERRUPT THE  
13 CONVERSATION. I JUST WANTED TO CLARIFY FOR BETH AND  
14 THE RECORD THAT THIS IS A MOTION TO FUND APPLICATION  
15 11165.

16 MR. SHEEHY: CHAIRMAN THOMAS HAD A  
17 COMMENT, AND THEN I HEARD MR. ROWLETT, AND THERE MAY  
18 BE OTHERS.

19 CHAIRMAN THOMAS: THANK YOU, MR. SHEEHY.  
20 SO I WOULD TEND TO BE SUPPORTIVE OF THIS FOR THE  
21 REASONS MENTIONED. AND, IN ADDITION, GIVEN THAT THE  
22 CHALLENGES OF DEMENTIA ARE MANY AND SIGNIFICANT,  
23 THAT ANYTHING GLEANED THROUGH THIS PARTICULAR LINE  
24 OF RESEARCH, I THINK, WOULD HAVE A SHOT OF INFORMING  
25 RESEARCH IN OTHER FORMS OF DEMENTIA. THIS, OF

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1 COURSE, IS CLOSELY RELATED TO ALZHEIMER'S, FOR  
2 EXAMPLE. AND I THINK THAT, GIVEN THE DIFFICULTY IN  
3 COMING UP WITH SOMETHING TO ADDRESS ANY OF THESE  
4 CONDITIONS HAS BEEN SO PRONOUNCED, THAT WE WOULD BE  
5 WISE TO FUND SOMETHING IN THIS SPACE.

6 HAVING SAID THAT, I WOULD LIKE TO ASK DR.  
7 SAMBRANO OR DR. PATEL. THIS DID GET FIVE TIER IIS.  
8 IF YOU COULD JUST INFORM THE BOARD ON THE REASONS  
9 FOR THOSE VOTES, THAT WOULD BE HELPFUL. THANK YOU.

10 DR. PATEL: CERTAINLY. WOULD YOU LIKE ME  
11 TO GIVE YOU AN OVERVIEW OF THE PROPOSAL AS WELL, OR  
12 WOULD YOU JUST LIKE TO KNOW --

13 CHAIRMAN THOMAS: SURE. I THINK THAT  
14 WOULD BE HELPFUL FOR THE BOARD.

15 DR. PATEL: OKAY. SO THIS ONE IS AN  
16 APPLICATION THAT'S FOCUSED ON PROGRANULIN DEFICIENT  
17 FRONTOTEMPORAL DEMENTIA. SO IT IS A SUBSET OF WHAT  
18 IS THE SECONDMOST COMMON DEMENTIA FOR THOSE UNDER  
19 65. SO THE FRONTAL LENS IS ABOUT 15 TO 20 PER  
20 100,000 INDIVIDUALS.

21 THERE'S A FAMILIAL TYPE, WHICH IS THIS  
22 ONE, WHICH IS THE PROGRANULIN MUTATION. THERE'S A  
23 LOSS OF FUNCTION THAT RESULTS IN THIS PARTICULAR  
24 TYPE OF DEMENTIA THAT THIS APPLICATION ADDRESSES.

25 SO WHAT THEY INTEND TO DO IS UTILIZE

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1 INDUCED PLURIPOTENT STEM CELLS TO GENERATE  
2 MICROGLIA, WHICH IS A MACROPHAGE IN THE NEURAL  
3 SYSTEM -- IT'S AN IMMUNE CELL SYSTEM -- AND USE  
4 THOSE TO MAKE THEM TRANSPLANTABLE IN ORDER TO  
5 RESTORE THE PROGRANULIN AS WELL AS TEST THE EFFECT  
6 ON MOUSE MODELS OF DEMENTIA. SO THAT'S WHAT THEY  
7 INTEND TO DO WITH THIS WITH THE HOPE OF DEVELOPING A  
8 THERAPY, A CELL THERAPY, THAT COULD ULTIMATELY  
9 IMPACT ON THOSE PATIENTS.

10 SO AS MENTIONED BEFORE, THE DISEASE IS NOT  
11 REPRESENTED CURRENTLY IN OUR PORTFOLIO. ALTHOUGH  
12 THERE IS ONE AWARD TO DEVELOP A METHOD FOR  
13 DIFFERENTIATING PLURIPOTENT STEM CELLS INTO  
14 MICROGLIA.

15 IN TERMS OF THE WORKING GROUP  
16 RECOMMENDATION, IN THE CONTEXT OF ALL OF THESE  
17 APPLICATIONS THAT YOU ARE LISTENING TO HAVING HAD A  
18 POSITIVE VIEW BY THE GWG, THERE ARE ONLY A FEW  
19 COMMENTS THAT MIGHT CAPTURE WHAT SOME OF THESE GWG  
20 REVIEWERS MAY HAVE THOUGHT WAS A CONCERN.

21 SO FOR THIS APPLICATION, IT WAS MENTIONED  
22 THAT IT WAS UNCLEAR WHETHER THERE WOULD BE ENOUGH  
23 CELLS OR THE ABILITY TO SCALE UP THE NUMBER OF CELLS  
24 THAT WOULD BE REQUIRED FOR A HUMAN THERAPY. BUT ON  
25 THE OTHER HAND, THIS IS AN APPLICATION THAT IS AT

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1 THE STAGE WHERE IT'S DEVELOPING ITS PROOF OF  
2 PRINCIPLE. SO ALTHOUGH A CONCERN, IT'S SOMETHING  
3 THAT THE APPLICANTS WOULD BE WORKING TOWARDS IN  
4 TERMS OF ACHIEVING. THAT WAS THE MOST SALIENT  
5 COMMENT THAT WAS MADE BY GWG IN TERMS OF CONCERN.

6 MR. SHEEHY: DO WE HAVE OTHER QUESTIONS OR  
7 COMMENTS FROM OTHER --

8 MS. MILLER: THIS IS LAUREN. I JUST  
9 WANTED TO ADD THAT THE ALZHEIMER'S AND DEMENTIA  
10 COMMUNITY, AS I THINK SO MANY OF YOU KNOW, ARE  
11 DESPERATE FOR INNOVATION. AND THERE ARE SO FEW  
12 INNOVATIVE STUDIES LIKE THIS THAT ARE HAPPENING OUT  
13 THERE, AND THAT FUNDING SOMETHING LIKE THIS WOULD  
14 GIVE SUCH ENERGY TO ALL DEMENTIA PATIENT ADVOCATES  
15 AND, THEREFORE, I THINK, WOULD CREATE SUCH AN  
16 EXCITEMENT OVER THE WORK THAT CIRM IS DOING IN  
17 ADDITION TO POTENTIALLY CREATING A TREATMENT FOR  
18 SOMETHING THAT, AS A FEW PEOPLE HAVE TOUCHED ON, IS  
19 EXTRAORDINARILY COSTLY, THE MOST COSTLY DISEASE IN  
20 THIS STATE AND THE COUNTRY, AND IS GROWING  
21 ASTRONOMICALLY AS FAR THE NUMBER OF PATIENTS AND IS,  
22 AS I SAID, DESPERATE FOR SOMETHING TO MOVE THE  
23 NEEDLE. AND I THINK THAT CIRM FUNDING SOMETHING  
24 LIKE THIS WOULD BE TREMENDOUSLY HELPFUL FOR BOTH THE  
25 DISEASE AND CIRM OVERALL.

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1 DR. MARTIN: I HAVE A TECHNICAL QUESTION  
2 ABOUT THE PROPOSAL. ANIMAL MODELS OF THESE TYPES OF  
3 DISEASES ARE VERY DIFFICULT IF THEY'RE AT ALL REAL.  
4 SINCE THE SELECTION OF A HUMAN DISEASE IS AN  
5 INHERITED FRONTOTEMPORAL DEMENTIA, IS THE MOUSE  
6 MODEL A TRANSGENIC GENETIC MODEL OF THE SAME GENE  
7 DEFECT?

8 DR. PATEL: LET ME LOOK THAT UP. I  
9 BELIEVE IT IS. IT'S A MODEL WHERE THE PROGRANULIN  
10 IS DEFICIENT. LET ME LOOK THAT UP TO GIVE YOU A  
11 DEFINITIVE ANSWER ON THAT.

12 DR. MARTIN: MY COMMENT WOULD BE THAT THAT  
13 MAY BE A SIGNIFICANT ADVANTAGE FOR THIS MODEL FOR  
14 THIS DIFFICULT DISEASE BECAUSE THERE IS VERY LIKELY  
15 A REASONABLE PHENOTYPE IN THAT MOUSE THAT CAN BE  
16 MONITORED; WHEREAS, OTHER MOUSE MODELS FOR CNS  
17 DISEASES, UNLESS THEY'RE TRANSGENIC AND OF THE SAME  
18 GENE, ARE USUALLY NOT RELIABLE AND NOT PREDICTIVE.

19 MR. SHEEHY: WHILE WE WAIT FOR DR.  
20 SAMBRANO, ARE THERE OTHER COMMENTS? I NOTE MR.  
21 ROWLETT HAD A COMMENT THAT HE WANTED TO MAKE OR A  
22 QUESTION.

23 MR. ROWLETT: SOMETIMES THERE'S AN  
24 ADVANTAGE -- THIS IS AL ROWLETT -- TO LISTENING TO  
25 OTHERS. AS THE PATIENT ADVOCATE FOR THIS AREA, I

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1 CERTAINLY ENDORSE ALL THE ADVOCACY AND APPRECIATE  
2 THE COMMENTS.

3 I THINK, AGAIN, HAVING THIS BE THE FIFTH  
4 GRANT THAT WE WOULD APPROVE TODAY, CERTAINLY I WOULD  
5 SUPPORT THAT GIVEN THE OTHER FOUR RECEIVED NO  
6 UNFUNDABLE SCORES FROM ANY OF THE REVIEWERS AND ALSO  
7 GIVEN THE COMMENTS BY DR. SAMBRANO REGARDING WHAT  
8 THE REVIEWERS SAID THAT ULTIMATELY ENDED UP THIS  
9 BEING A TIER II. SO NOT TO REPEAT MYSELF, BUT I  
10 ALSO SUPPORT. AND THANK YOU VERY MUCH, SENATOR, FOR  
11 MOVING THIS UP.

12 MR. TORRES: THANK YOU.

13 DR. SAMBRANO: JUST TO ANSWER THE PREVIOUS  
14 QUESTION ON THE MOUSE MODEL. SO THIS IS A GRN MINUS  
15 MINUS MOUSE, SO IT IS A TRANSGENIC THAT HAS THE  
16 PROGRANULIN KNOCKED OUT, AND THEY WILL COMPARE IT TO  
17 THE WILD TYPE.

18 DR. MARTIN: THANK YOU. THAT'S  
19 ENCOURAGING.

20 MR. SHEEHY: OTHER QUESTIONS AND COMMENTS?

21 MR. TORRES: CALL FOR THE QUESTION.

22 MR. SHEEHY: ABSOLUTELY. IS THERE PUBLIC  
23 COMMENT AT ANY OF THE SITES? SEEING NO PUBLIC  
24 COMMENT, COULD WE CALL THE ROLL.

25 MS. BONNEVILLE: DAVID HIGGINS.

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1 DR. HIGGINS: YES.  
2 MS. BONNEVILLE: STEVE JUELSGAARD.  
3 MR. JUELSGAARD: YES.  
4 MS. BONNEVILLE: DAVE MARTIN.  
5 DR. MARTIN: YES.  
6 MS. BONNEVILLE: LAUREN MILLER.  
7 MS. MILLER: YES.  
8 MS. BONNEVILLE: ADRIANA PADILLA.  
9 DR. PADILLA: 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 MR. SHEEHY: YES.  
18 MS. BONNEVILLE: OS STEWARD.  
19 DR. STEWARD: YES.  
20 MS. BONNEVILLE: JONATHAN THOMAS.  
21 CHAIRMAN THOMAS: YES.  
22 MS. BONNEVILLE: ART TORRES.  
23 MR. TORRES: AYE.  
24 MS. BONNEVILLE: DIANE WINOKUR.  
25 MS. WINOKUR: YES.

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1 MS. BONNEVILLE: THANK YOU. THE MOTION  
2 CARRIES.

3 MR. SHEEHY: OKAY. DO WE HAVE AN  
4 ADDITIONAL MOTION ON AN APPLICATION?

5 I ACTUALLY AM GOING TO STEP IN AND SUGGEST  
6 11107. AND THE REASON IS BECAUSE IT'S OVARIAN  
7 CANCER, WHICH WE'VE DONE -- ACTUALLY I DON'T THINK  
8 WE'VE DONE ANY WORK IN OVARIAN CANCER. WE DID A  
9 COUPLE OF DISCOVERY, BUT WE MADE NO PROGRESS. AND  
10 THIS IS A CANCER THAT IS JUST HORRIFYING. AND  
11 HAVING SEEN PEOPLE WHO SUFFERED FROM OVARIAN CANCER,  
12 THERE'S REALLY NO TREATMENT. IT SEEMS LIKE A GREAT  
13 TARGET FOR CAR T CELL THERAPY BY A GROUP THAT'S  
14 REALLY GOOD AT CAR T CELL THERAPY AND DEVELOPING  
15 NOVEL CAR T THERAPIES. AND I THINK THE IMPACT WOULD  
16 BE HUGE ON A CANCER THAT IMPACTS MANY, MANY PEOPLE  
17 IN CALIFORNIA, MANY WOMEN IN CALIFORNIA. SO I MAKE  
18 THAT MOTION IF THERE'S A SECOND.

19 MR. JUELSGAARD: SECOND.

20 MR. SHEEHY: THERE'S A SECOND.  
21 DISCUSSION? ANY PUBLIC COMMENT?

22 DR. PRICEMAN: MEMBERS OF THE BOARD, MY  
23 NAME IS SAUL PRICEMAN FROM THE CITY OF HOPE, AND I'M  
24 THE PRINCIPAL INVESTIGATOR FOR THE APPLICATION  
25 DISC1-11107. I ALSO SUBMITTED ONE OF THE LETTERS



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1 THAT HAS BEEN POSTED FOR PUBLIC REVIEW.

2 EVERY SINGLE DAY FIVE WOMEN IN CALIFORNIA  
3 WILL BE DIAGNOSED WITH OVARIAN CANCER. FOUR OUT OF  
4 THESE FIVE WOMEN WILL HAVE LATE STAGE, AGGRESSIVE,  
5 AND INCURABLE DISEASE, AND ONLY ONE WILL SURVIVE  
6 BEYOND FIVE YEARS. WE ALL KNOW SOMEONE WHO HAS BEEN  
7 AFFLICTED WITH THIS DREADED DISEASE. IN FACT, IN  
8 THE UNITED STATES, 22,000 WOMEN WILL BE DIAGNOSED  
9 WITH IT THIS YEAR. AND OF THESE, 14,000 WILL DIE.

10 IN CHECKING THE CIRM WEBSITE, I WAS  
11 SURPRISED TO FIND THAT CIRM HAS FUNDED ONLY ONE  
12 GRANT EXCLUSIVELY ON OVARIAN CANCER. THIS IS AN  
13 EARLY STAGE DISC1 AWARD AT THE COST OF \$172,000 FOR  
14 RESEARCH. IT MAKES OVARIAN CANCER AN  
15 UNDERREPRESENTED DISEASE IN TERMS OF CIRM FUNDING.

16 WE HOPE THAT YOU WILL CHANGE THIS BECAUSE  
17 WE BELIEVE OUR APPROACH, A CAR T CELL STRATEGY, WILL  
18 REVOLUTIONIZE THE TREATMENT FOR WOMEN WITH OVARIAN  
19 CANCER. BECAUSE WE'RE VERY ANXIOUS TO GET TO  
20 CLINICAL TRIAL, WE PROVIDE A CLEAR AND ACCELERATED  
21 MILESTONE THAT LEADS TO PRE-FDA FILING AT THE END OF  
22 THE TWO-YEAR FUNDING PERIOD. THIS MAY SEEM  
23 AMBITIOUS FOR A QUEST AWARD, BUT WORKING AT CITY OF  
24 HOPE, AS WAS MENTIONED, MAKES A HUGE DIFFERENCE TO  
25 THE FEASIBILITY OF OUR TIMELINE.

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1 CITY OF HOPE HAS FOCUSED A GREAT DEAL OF  
2 RESOURCES ON THE DEVELOPMENT OF NEW CAR APPROACHES  
3 FOR TREATING THE MOST INCURABLE AND DEADLY SOLID  
4 TUMORS. IN THE EARLY 2000S, CITY OF HOPE WAS THE  
5 FIRST TO TREAT SOLID TUMOR PATIENTS WITH CAR T CELLS  
6 AND IS RECENTLY THE FIRST TO DEMONSTRATE A DURABLE  
7 CLINICAL RESPONSE IN A PATIENT WITH GLIOBLASTOMA.

8 BY THE END OF 2018, WE WILL BE ENROLLING  
9 PATIENTS ON OUR ONGOING TRIAL FOR GLIOBLASTOMA, AND  
10 WE WILL INITIATE TRIALS FOR BOTH PROSTATE CANCER AND  
11 BREAST CANCER WITH CAR T CELL THERAPIES. OVARIAN  
12 CANCER IS THE NEXT HURDLE WE AIM TO TACKLE. WE DO  
13 UNDERSTAND THE CHALLENGES FACING THE DEVELOPMENT OF  
14 EFFECTIVE CAR T CELL THERAPIES FOR SOLID TUMORS AND,  
15 IN PARTICULAR, THE HARSH ENVIRONMENT CREATED BY THE  
16 TUMOR HAS HISTORICALLY PREVENTED IMMUNOTHERAPY FROM  
17 WORKING FOR THESE PATIENTS.

18 TO ADDRESS THESE CHALLENGES, WE WILL  
19 ENHANCE THE STEMNESS OF T-CELLS SO THAT THEY WILL BE  
20 ABLE TO RETAIN IN THE BODY FOR A LONG PERIOD OF  
21 TIME. WE ALSO PROMOTE THEIR TRAFFICKING TO THE  
22 SOLID TUMORS AS WELL AS IMPROVE THEIR ABILITY TO  
23 KILL TUMORS ONCE THEY ARRIVE THERE. THESE  
24 APPLICATIONS HAVE BROAD STROKES ACROSS ALL SOLID AND  
25 LIQUID SOLID TUMORS.

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1           IN SUMMARY, WE HOPE YOU WILL FUND OUR  
2 PROPOSAL AND LET US MOVE SWIFTLY TO CONDUCT THE  
3 NECESSARY STUDIES TO PRESENT TO THE FDA. THANK YOU.

4           MR. SHEEHY: THANK YOU. IS THERE  
5 ADDITIONAL PUBLIC COMMENT? CAN WE CALL THE ROLL.

6           MS. BONNEVILLE: DAVID HIGGINS. STEVE  
7 JUELSGAARD.

8           MR. JUELSGAARD: YES.

9           MS. BONNEVILLE: DAVE MARTIN.

10          DR. MARTIN: YES.

11          MS. BONNEVILLE: LAUREN MILLER.

12          MS. MILLER: YES.

13          MS. BONNEVILLE: ADRIANA PADILLA.

14          DR. PADILLA: 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          MR. SHEEHY: YES.

23          MS. BONNEVILLE: OS STEWARD.

24          DR. STEWARD: YES.

25          MS. BONNEVILLE: JONATHAN THOMAS.

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1 CHAIRMAN THOMAS: YES.

2 MS. BONNEVILLE: ART TORRES.

3 MR. TORRES: AYE.

4 MS. BONNEVILLE: DIANE WINOKUR.

5 MS. WINOKUR: YES.

6 MS. BONNEVILLE: THE MOTION CARRIES.

7 MR. SHEEHY: GREAT. SO CAN WE GET AN  
8 UPDATE ON FUNDS AVAILABLE?

9 DR. SAMBRANO: SURE. SO WHAT REMAINS AT  
10 THIS POINT IS 1.7 MILLION. THAT WOULD COVER  
11 VIRTUALLY ANY AWARD EXCEPT FOR ONE IN THE TOP TIER.

12 CHAIRMAN THOMAS: A QUESTION FOR DR.  
13 SAMBRANO OR THE TEAM. HOW MANY PROJECTS HAVE WE  
14 FUNDED FOR ANY FIBROSIS RELATED? I SEE THAT WE'VE  
15 GOT A PULMONARY FIBROSIS ON HERE. IT REFERENCES A  
16 COUPLE OF AWARDS FOR PULMONARY AND ONE FOR CYSTIC  
17 FIBROSIS. IS THAT THE EXTENT OF OUR FIBROSIS  
18 PORTFOLIO?

19 DR. SAMBRANO: SO WHAT WE REPRESENT THERE  
20 ARE ACTIVE AWARDS. ONE AWARD, AS YOU NOTED, FOR  
21 CYSTIC FIBROSIS AND TWO THAT WOULD IMPACT ON THE  
22 IDIOPATHIC PULMONARY FIBROSIS, WHICH IS THE SUBJECT  
23 OF THE APPLICATION BEFORE US.

24 CHAIRMAN THOMAS: AND IS THERE A THEORY  
25 THAT IF YOU WERE ABLE TO MAKE HEADWAY ON ONE

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1 PARTICULAR FIBROTIC CONDITION, THAT IT COULD  
2 POSSIBLY HELP INFORM RESEARCH ON OTHERS?

3 DR. SAMBRANO: SO THE CLAIM BY THE  
4 APPLICANTS FOR THIS APPLICATION IS THAT IT COULD.  
5 THERE ARE DIFFERENT TYPES OF FIBROSIS, NOT  
6 NECESSARILY CYSTIC FIBROSIS, WHICH IS ONE THAT COULD  
7 POSSIBLY, BUT THERE ARE DIFFERENT STRATEGIES FOR  
8 ADDRESSING CYSTIC FIBROSIS.

9 CHAIRMAN THOMAS: SO ON THE THEORY THAT  
10 ONE CRITERION THAT WE MIGHT APPLY IS FUNDING  
11 PROJECTS THAT COULD POTENTIALLY LEAD TO AN IMPACT ON  
12 MORE THAN ONE CONDITION, AND NOTING THAT, AS IS THE  
13 CASE WITH EVERYTHING WE CONSIDER HERE, OF COURSE,  
14 THE PULMONARY FIBROSIS AND CYSTIC FIBROSIS ARE BOTH  
15 TERRIBLE DISORDERS AND THAT WE DON'T HAVE A WHOLE  
16 LOT IN THE PORTFOLIO ADDRESSING THESE, I WOULD MOVE  
17 THAT WE APPROVE ITEM 11192 FOR APPROVAL.

18 DR. JUELSGAARD: I'LL SECOND THAT MOTION.

19 MR. SHEEHY: IS THERE ANY COMMENT?

20 DR. MARTIN: THIS IS DAVE. I WOULD JUST  
21 SO WE UNDERSTAND, CYSTIC FIBROSIS AND WHAT IS CALLED  
22 PULMONARY FIBROSIS HAVE COMPLETELY DIFFERENT  
23 MECHANISMS OF PATHOGENESIS. COMPLETELY DIFFERENT.

24 CHAIRMAN THOMAS: THAT'S WHY I WAS ASKING  
25 THE QUESTION TO DR. SAMBRANO. IS THERE A CHANCE

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1 THAT -- AND OBVIOUSLY THE WAY THE CONDITIONS PLAY  
2 OUT IS DIFFERENT AS WELL. PULMONARY FIBROSIS COULD  
3 BE IN ITS MOST VIRULENT FORM FATAL IN SIX MONTHS.  
4 THERE ARE LONGER LASTING VERSIONS OF IT THAT CAN  
5 SPAN OVER YEARS; BUT, NONETHELESS, HAVE A  
6 HORRIBLE -- BUT, DAVE, THAT'S EXACTLY WHY I ASKED  
7 THAT QUESTION.

8 DR. MARTIN: I'M JUST POINTING OUT THAT  
9 YOU'RE OBVIOUSLY CORRECT ON THAT. I'M JUST SAYING  
10 THAT CYSTIC FIBROSIS SHOULD BE JUST BE SORT OF OUT.  
11 IT'S A COMPLETELY DIFFERENT DISEASE. SO THERE WON'T  
12 BE CROSSOVER THERE I'M RELATIVELY CERTAIN, AS  
13 CERTAIN AS ONE CAN BE ABOUT ANYTHING GOING ON IN THE  
14 FORM OF FIBROSIS, BUT THERE ARE PLENTY -- THERE IS,  
15 AS YOU POINT OUT, CLEAR HETEROGENEITY WITHIN THE  
16 GENERAL CLASS OF PULMONARY FIBROSIS.

17 MR. SHEEHY: ADDITIONAL COMMENTS? SO JUST  
18 TO NOTE THAT THIS WILL BE THE LAST ONE THAT WE'RE  
19 ABLE TO APPROVE. SO IF ANYONE HAS ANY INTEREST IN  
20 ANY OTHER APPLICATIONS, YOU MIGHT NOTE THAT.

21 I WILL SAY THAT FOR ME I DO LOOK AT 11109  
22 BECAUSE THERE'S NOTHING ELSE FOR THAT INDICATION.  
23 IT'S A VERY RARE DISEASE AND UNLIKELY TO GET SUPPORT  
24 FROM -- I THINK -- IS THIS AN HSCC? IS THIS AN  
25 EMBRYONIC STEM CELL-DERIVED THERAPY? THIS IS AN

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1 IPS-DERIVED THERAPY WHICH ALIGNS WITH CIRM'S  
2 MISSION.

3 SO TO ME --

4 DR. JUELSGAARD: CALL THE QUESTION.

5 MR. SHEEHY: CALL THE QUESTION? OKAY.

6 ANY PUBLIC COMMENT AT ANY SITE? WE HAVE PUBLIC  
7 COMMENT HERE IN SAN FRANCISCO. JUST IDENTIFY  
8 YOURSELF.

9 DR. WEINACHT: THANK YOU SO MUCH FOR  
10 GIVING ME THE OPPORTUNITY TO SPEAK. MY NAME IS  
11 KATJA WEINACHT. I'M A PHYSICIAN SCIENTIST AND STEM  
12 CELL TRANSPLANTER AT STANFORD SCHOOL OF MEDICINE AND  
13 TOGETHER WITH MY COLLABORATOR, VITTORIO SEBASTIANO,  
14 WHO IS A DEVELOPMENTAL BIOLOGIST, AND WE PROPOSE TO  
15 YOU TO MAKE REGENERATIVE THYMIC TISSUE TO CURE THE  
16 IMMUNE DEFECT IN A DISEASE CALLED 22Q11 SYNDROME.

17 AS YOU POINTED OUT, THE MISSION OF CIRM IS  
18 TO FUND INCURABLE DISEASES AND FIND THERAPIES FOR  
19 DISEASES WHERE THERE'S NOTHING OUT THERE. AND IT IS  
20 EXACTLY THE 22Q11 DISEASE. IT'S A MULTICENTRIC  
21 DISEASE, BUT WHAT KILLS THESE PATIENTS IS THAT THEY  
22 DO NOT HAVE A THYMUS AND, THEREFORE, THEY DO NOT  
23 HAVE AN IMMUNE SYSTEM. AND CURRENTLY TO THIS DATE  
24 THERE IS NO THERAPY OUT THERE. SO THESE PATIENTS  
25 ARE CONFINED IN THE ICU IN A PROTECTED ENVIRONMENT

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1 INEVITABLY UNTIL THEY CATCH A LETHAL INFECTION AND  
2 THEREFORE DIE.

3 SO WHAT WE ARE PROPOSING TO YOU AND THE  
4 SCOPE OF OUR PROPOSAL IS TO MAKE REGENERATIVE THYMIC  
5 TISSUES FOR INDUCED PLURIPOTENT STEM CELLS. THE  
6 GOAL OF THIS PROPOSAL IS TWOFOLD. FIRST, WE CAN  
7 MAKE SCALABLE TISSUES IN WHATEVER AMOUNT FOR ALL THE  
8 PATIENTS WHO NEED THEM. WE HAVE TISSUES THAT ARE  
9 HLA-COMPATIBLE OR PATIENT SPECIFIC AND THEY'RE  
10 SUPERIOR TO THE FORMERLY AVAILABLE TRANSPLANT AND IS  
11 NO LONGER AVAILABLE TO DATE.

12 I THINK EVERYBODY (UNINTELLIGIBLE) CIRM  
13 PORTFOLIO, YOU HAVE IN THE PAST FIVE APPLICATIONS  
14 THAT SUPPORTED THYMUS-RELATED OR THYMIC  
15 FUNCTION-RELATED PROJECTS, BUT YOU'VE NEVER HAD A  
16 PROJECT THAT ACTUALLY WANTED TO MAKE REGENERATIVE  
17 THYMIC TISSUES THAT REPLACE THE THYMIC FUNCTION OF  
18 PATIENTS WHO DO NOT HAVE A THYMUS AT ALL.

19 (UNINTELLIGIBLE) CIRM GIVE US A WONDERFUL  
20 INFRASTRUCTURE FOR US TO BUILD ON RIGHT NOW. WE  
21 HAVE THE TECHNOLOGY TO REALLY PUT IPS-DERIVED  
22 THERAPIES INTO THE CLINIC VERY QUICKLY. AND I THINK  
23 IF THIS GRANT GETS FUNDED, WITHIN TWO YEARS WE MAY  
24 BE ABLE TO FILE A PRE-IND, WE MAY BE ABLE TO MOVE TO  
25 A CLINICAL DESIGN, AND WE MAY BE ABLE TO START



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1 TREATING AND CURING THESE PATIENTS.

2 I THINK WE OWE THIS TO OUR PATIENTS AND  
3 THE FAMILY, AND WE HAVE A MOTHER ON THE PHONE. AND  
4 IF AT ALL POSSIBLE, I WOULD LOVE IT IF YOU COULD  
5 HEAR FROM KATIE WILKERSON WHOSE SON IS SEVEN MONTHS  
6 SINCE HE WAS BORN.

7 MR. SHEEHY: YES. IS THERE SOMEONE ON THE  
8 PHONE THAT WOULD LIKE TO MAKE A COMMENT?

9 MS. CHEUNG: KATIE, IF YOU ARE ON THE  
10 LINE, IF YOU CAN HIT STAR ONE.

11 MS. WILKERSON: MY NAME IS KATIE  
12 WILKERSON. I AM THE MOM OF AN AMAZING  
13 SEVEN-AND-A-HALF-MONTH-OLD LITTLE BOY, CHARLIE, WHO  
14 WAS BORN WITHOUT A THYMUS.

15 I LIVE MY LIFE IN CONSTANT FEAR OF WHAT  
16 EVERY COUGH, EVERY SNEEZE, EVERY CRY WILL BRING.  
17 EACH PERSON THAT COMES INTO MY SON'S ROOM, I WORRY  
18 THAT THIS WILL BE THE LAST TIME HE GETS A VIRUS AND  
19 WE'RE GOING TO LOSE HIM.

20 MY SIX-YEAR-OLD FREQUENTLY ASKS WHAT WILL  
21 HAPPEN IF CHARLIE DIES? THIS BREAKS MY HEART  
22 BECAUSE I CAN'T EVEN TELL HIM THAT WE'LL HAVE AND BE  
23 PROVIDED A CURE. THIS POSSIBILITY IS ALL TOO REAL.  
24 I WISH I COULD SAY I'M ALONE IN THIS, BUT I'VE  
25 GOTTEN TO KNOW TOO MANY PARENTS IN THE SAME

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1 PREDICAMENT.

2 WHEN CHARLIE WAS ONE WEEK OLD, MY FAMILY'S  
3 WORLD COMPLETELY CHANGED WHEN THE NEWBORN SCREENING  
4 CAME BACK SHOWING CHARLIE HAD NO T CELLS. AT THE  
5 TIME WE THOUGHT HE HAD SCID AND WE WERE OVERWHELMED.  
6 AS WE TALKED WITH DOCTORS, THEY ASSURED US THERE  
7 WERE TREATMENT OPTIONS. CHARLIE WAS HOSPITALIZED IN  
8 ISOLATION BECAUSE I HAVE THREE OTHER SMALL KIDS WHO  
9 ARE GERM BALLS BEING 6, 5, AND 3 YEARS OLD. IF WE  
10 HAD BEEN TOLD AT THE TIME WHAT WE HAD IN STORE, I  
11 THINK I WOULD HAVE BEEN COMPLETELY CRUMBLLED UNDER  
12 THE REALITY OF IT ALL.

13 AFTER SEVERAL MORE TESTS, WE WERE GIVEN  
14 THE NEWS THAT CHARLIE DID NOT HAVE SCID, BUT HAD  
15 COMPLETE DIGEORGE. I WENT INTO A RESEARCH FRENZY  
16 LEARNING ANYTHING I COULD. I WANTED TO BECOME  
17 KNOWLEDGEABLE ON CHARLIE'S CONDITION BECAUSE MANY OF  
18 CHARLIE'S DOCTORS HAD NEVER TREATED A COMPLETE  
19 DIGEORGE PATIENT. THEY HAD NEVER SEEN THE CONDITION  
20 BEFORE. I WAS TOLD THE ONLY TREATMENT OPTION FOR  
21 HIM WAS A THYMUS TRANSPLANT AT DUKE UNIVERSITY WHICH  
22 IS STILL IN A RESEARCH PHASE. WE RUSHED TO GET ALL  
23 THE INFORMATION THEY REQUIRED TO ADD HIM TO THEIR  
24 RESEARCH STUDY AS SOON AS POSSIBLE. OUR PACKET WAS  
25 SENT MIDDLE OF JANUARY. MONTH AFTER MONTH OUR

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1 INFORMATION WAS NOT REVIEWED AND THEY WERE NOT  
2 TAKING PATIENTS FOR TRANSPLANT.

3 SEVERAL MONTHS LATER THEY SENT OUT A  
4 LETTER TO PATIENTS ON THE WAIT LIST, WHICH WE HAD  
5 NOT EVEN GOTTEN TO THAT YET, SAYING THEIR PROGRAM  
6 WAS SUSPENDED. MY HEART COMPLETELY DROPPED. WITH  
7 NO KIND OF INFORMATION ON WHEN THEY WOULD REVIEW, I  
8 FELT LIKE MY SON'S CHANCE OF LIFE WAS ON HOLD. HE  
9 IS KEPT IN ISOLATION IN THE HOSPITAL, AND WE HAVE  
10 BEEN ABLE TO KEEP HIM VIRUS FREE. YET EACH DAY  
11 REMAINS A GAME OF RUSSIAN ROULETTE. IS TODAY THE  
12 DAY THAT CHARLIE GETS SICK?

13 MY TIME IS SPLIT BETWEEN MY THREE KIDS AT  
14 HOME AND MY BOY IN THE HOSPITAL. MY HUSBAND IS  
15 ACTIVE DUTY IN THE MILITARY AND HASN'T BEEN THERE,  
16 LEAVING ME TO BE THE SOLE CARE PROVIDER FOR OUR FOUR  
17 CHILDREN.

18 IF DUKE RESUMES THEIR TRANSPLANTS, THERE  
19 ARE DOZENS AHEAD OF CHARLIE. I WILL DO EVERYTHING  
20 IN MY POWER TO KEEP MY SON HEALTHY TO GET THERE, BUT  
21 WORRY IS THIS REALLY POSSIBLE FOR ANOTHER YEAR OR  
22 MORE. EVEN IF HE GETS A TRANSPLANT, IT ISN'T A  
23 PERFECT FIT BECAUSE THEY ARE NOT HLA-MATCHED. SO  
24 HIS CHANCE OF AUTOIMMUNE ISSUES DRASTICALLY GO UP.

25 I IMPLORE YOU TO FUND DR. WEINACHT AND DR.

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1 SEBASTIANO'S RESEARCH FOR REGENERATIVE THYMUS  
2 TISSUE. NOT ONLY DOES MY SON'S LIFE LITERALLY  
3 DEPEND ON IT, BUT SO DO DOZENS OF OTHER KIDS TODAY  
4 AND IN THE FUTURE. IN ORDER TO LIVE, CHARLIE NEEDS  
5 A THYMUS. THANK YOU.

6 MR. SHEEHY: THANK YOU. CHAIRMAN THOMAS.

7 CHAIRMAN THOMAS: SO THANK YOU FOR THAT  
8 TESTIMONY, BOTH OF YOU. BEFORE WE, AS WE DO HAVE A  
9 MOTION ON THE TABLE HERE, BEFORE WE VOTE ON THAT, I  
10 WANT TO REVISIT ONE OF MR. JUELSGAARD'S IDEAS AND  
11 PLAY DEVIL'S ADVOCATE TO OS AND HIS COMMENT IN  
12 RESPONSE TO THAT.

13 SO MR. JUELSGAARD SUGGESTED AS A  
14 POSSIBILITY MOVING TO FUND ALL OF THESE PROJECTS  
15 WITH THE CAVEAT THAT WE APPROVE A CERTAIN NUMBER  
16 TODAY AND THEN THE BALANCE WOULD BE AUTOMATICALLY  
17 FUNDED IN THE BEGINNING OF THE NEXT BUDGET CYCLE,  
18 WHICH WOULD BE THE FIRST OF THE YEAR. IN DOING  
19 THAT, WHAT YOU WOULD BE DOING IS AGREEING TODAY TO  
20 FUND WHAT WOULD BE A LIST OF ALL RECOMMENDED FOR  
21 FUNDING PROJECTS. AND WE ARE IN, AMONG OTHER  
22 THINGS, THE TIME BUSINESS HERE, WHICH IS TO SAY  
23 WE'RE -- IF WE DID NOT DO SOMETHING LIKE THAT AND  
24 WAITED UNTIL THE NEXT QUEST ROUND, AS I INDICATED,  
25 THAT'S GOING TO BE NEXT JUNE WITH APPROVALS NEXT

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1 JULY, SO YOU'D BE LOOKING AT A SIX-MONTH FURTHER  
2 DELAY IN FUNDING. AND YOU HAVE HERE BEFORE YOU A  
3 LIST OF PROJECTS THAT ARE ALL RECOMMENDED, AND WE  
4 JUST APPROVED ONE THAT WAS NO. 13 OR 14 OR WHATEVER  
5 ON THE LIST, SUGGESTING THE FACT THAT THE BOARD  
6 UNDERSTANDS FULLY THAT EACH OF THESE PROJECTS IS  
7 WORTHY OF FUNDING.

8 YES, IF WE WERE TO DO SOMETHING LIKE THIS,  
9 IT WOULD HAVE AN IMPACT ON BUDGET DISCUSSIONS. IF,  
10 FOR EXAMPLE, WE DECIDED IN THE NEXT BUDGET  
11 DISCUSSION ROUND THAT WE MIGHT WANT TO PUT MORE  
12 EMPHASIS ON OTHER PARTS OF THE RESEARCH SPECTRUM,  
13 ETC., BUT I FOR ONE AM SUPPORTIVE OF MR.  
14 JUELSGAARD'S IDEA HERE. AND I RAISE IT AT THIS  
15 POINT BECAUSE, IF WE DO VOTE ON ANY SUBSEQUENT  
16 PROJECT HERE, IT WILL CLOSE OUT THIS ROUND AND  
17 PRECLUDE FUNDING THESE OTHERS UNLESS THEY COME BACK  
18 AND APPLY IN A YEAR.

19 SO I JUST WANT TO REVISIT THAT. AND, OS,  
20 I WELCOME YOUR COMMENTS ON THAT AND ANYBODY ELSE WHO  
21 HAS A THOUGHT ON IT.

22 DR. STEWARD: THANK YOU, J.T. THIS IS OS,  
23 AND I WILL MAKE A COMMENT.

24 SO EFFECTIVELY WHAT THAT DOES WOULD BE TO  
25 USE UP ALL OF NEXT YEAR'S LIKELY FUNDING FOR THE

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1 DISCOVERY ROUNDS.

2 CHAIRMAN THOMAS: THAT'S CORRECT.

3 DR. STEWARD: AND I THINK FORECLOSING  
4 DISCOVERY IN THAT WAY COULD BE A HUGE MISTAKE  
5 BECAUSE AT EACH STAGE OF THE RESEARCH PROCESS, THERE  
6 ARE THINGS THAT ARE VERY GOOD AND VERY EXCITING AND  
7 WONDERFUL, BUT NEW THINGS ARE COMING ALONG. I THINK  
8 THAT THERE'S A GOOD CHANCE THAT NEXT YEAR WE'LL BE  
9 SEEING THINGS THAT SIMPLY DON'T EXIST RIGHT NOW, AND  
10 THAT COMPLETELY FORECLOSING THE NEXT YEAR'S  
11 DISCOVERY ROUNDS WOULD BE VERY UNFORTUNATE.

12 THE ONLY WAY THAT WE COULD AVOID THAT  
13 WOULD BE TO LITERALLY DOUBLE NEXT YEAR'S DISCOVERY  
14 BUDGET, WHICH WOULD DETRACT FROM THE OTHER FUNDING  
15 THAT WE HAVE AVAILABLE. WE ARE COMING TO THE END.  
16 AND AS WE GO FORWARD, WE'RE GOING TO HAVE TO MAKE  
17 HARDER AND HARDER DECISIONS, AND I THINK WE REALLY  
18 NEED TO DO THAT NOW. THANK YOU.

19 MR. SHEEHY: SO HERE WE ARE. THERE IS A  
20 MOTION ON THE TABLE. IF YOU WANT TO WITHDRAW THAT  
21 MOTION AND THEN MAKE ANOTHER MOTION.

22 CHAIRMAN THOMAS: OKAY. I'LL WITHDRAW  
23 THAT MOTION.

24 MR. SHEEHY: DOES THE SECOND AGREE WITH  
25 THAT? I DON'T REMEMBER WHO THE SECOND IT.

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1 DR. JUELSGAARD: I DO, YES.

2 MR. SHEEHY: THAT MOTION IS WITHDRAWN.

3 SO YOU WANT TO MAKE ANOTHER MOTION TO  
4 SUBSTITUTE? YOU ACTUALLY KIND OF WITHDREW WHAT I  
5 THOUGHT I REALLY WOULD LIKE TO FUND PERSONALLY THIS  
6 ONE APPLICATION THAT WE'VE HEARD ABOUT TODAY. I  
7 WOULD BE HAPPY TO PROPOSE TO FUND THE REST IN THE  
8 NEXT CYCLE, BUT I THINK THE NEED IS PRETTY DESPERATE  
9 RIGHT NOW FOR CHROMATIN 22Q11 DELETION SYNDROME.

10 DR. JUELSGAARD: SO I HAVE A  
11 SPLIT-THE-BABY-LIKE PROPOSAL, WHICH IS WE HAVE TWO  
12 PROJECTS THAT WE'VE TALKED ABOUT, PULMONARY FIBROSIS  
13 AND THIS DELETION SYNDROME, BOTH FROM MY POINT OF  
14 VIEW SOUND VERY WORTHY OF FUNDING, BUT WE COULDN'T  
15 FUND BOTH OF THEM RIGHT NOW. HOWEVER, WE COULD  
16 APPROVE BOTH OF THEM AND SIMPLY PUT ONE OF THE TWO  
17 THAT WE APPROVE ON THE SLOWER PAYMENT TRACK. IN  
18 OTHER WORDS, WHATEVER FUNDING WE APPROVE NEXT YEAR,  
19 THAT FIRST DOLLAR IS FOR THE PROJECT THAT WE DON'T  
20 HAVE ENOUGH MONEY FOR TODAY. SO INSTEAD OF  
21 APPROVING ALL OF THE ONES THAT ARE ON THIS LIST OF  
22 15, WE STAY WITH WHAT WE'VE APPROVED, AND THEN  
23 APPROVE TWO, PULMONARY FIBROSIS, THE DELETION  
24 SYNDROME, REALIZING THAT SOME OF NEXT YEAR'S MONEY  
25 IS GOING TO BE PUT TO ONE OF THOSE PROJECTS. SO I

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1 WOULD INTRODUCE THAT AS A MOTION.

2 CHAIRMAN THOMAS: QUESTION ON THAT. MR.  
3 JUELSGAARD, I DON'T KNOW HOW LONG THESE PROJECTS  
4 TAKE TO PLAY OUT. WE CAN ASK DR. SAMBRANO. WHAT'S  
5 THE LENGTH OF --

6 DR. SAMBRANO: THE AWARDS ARE TWO YEARS.

7 CHAIRMAN THOMAS: SO IF YOU WERE TO ADOPT  
8 YOUR APPROACH, IN THEORY, SINCE WE'RE SPLITTING  
9 BABIES HERE, YOU COULD FUND HALF OF EACH AND GET  
10 THEM STARTED SINCE THEY'RE TWO-YEAR AWARDS ANYWAY.  
11 NO, CAN'T DO IT?

12 DR. JUELSGAARD: THE OTHER POSSIBILITY,  
13 AND I'D HAVE TO ASK SOMEBODY, THE PERSON FROM  
14 STANFORD THAT SPOKE A LITTLE BIT AGO, BECAUSE AT  
15 LEAST IN CERTAIN SITUATIONS IN ACADEMIA OR IN THE  
16 RESEARCH AREA, IF YOU HAVE ASSURANCE OF FUNDING  
17 THAT'S COMING DOWN THE ROAD, THE INSTITUTION IS  
18 ASSURED THAT FUNDING IS COMING, IT'S JUST NOT COMING  
19 AS QUICKLY AS YOU LIKE, SOMETIMES INSTITUTIONS WILL  
20 FRONT THE MONEY, PROVIDE YOU SOME FUNDING TO GET YOU  
21 UP AND RUNNING, AND THEN GET RECOMPENSED FOR THAT  
22 WHEN THE ACTUAL FUNDING COMES THROUGH. IF THAT'S  
23 TRUE, AND I DON'T KNOW IF IN EITHER OF THESE CASES  
24 IT IS TRUE, THAT'S ONE WAY OF NOT HAVING TO WORRY  
25 ABOUT THE TIMING INTERVAL. THE INITIAL FUNDING



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1 WOULD ACTUALLY COME FROM THE INSTITUTION FOR THE  
2 RESEARCH THAT'S BEING DONE, AND THEN THERE WOULD BE  
3 A MAKEUP THAT HAPPENS WHEN MONEY BECOMES AVAILABLE  
4 FROM CIRM GOING BACK TO THE INSTITUTION WHERE THE  
5 MONEY IS ALREADY INVESTED.

6 DO YOU KNOW WHETHER THAT'S A  
7 POSSIBILITY -- I'M SORRY. I DON'T REMEMBER YOUR  
8 NAME. IS THAT A POSSIBILITY AT STANFORD?

9 DR. WEINACHT: WHAT IS THE TIMELINE IN  
10 WHICH WE THINK THE FUNDING WOULD BECOME AVAILABLE?

11 DR. JUELSGAARD: MY UNDERSTANDING, AND I  
12 COULD BE CORRECTED, BUT IF WE DO WHAT I PROPOSE, THE  
13 ADDITIONAL MONEY WOULD BECOME AVAILABLE JANUARY 1ST.  
14 SO WE'RE TALKING ABOUT FIVE MONTHS FROM NOW, SO A  
15 RELATIVELY COMPRESSED TIME PERIOD.

16 DR. WEINACHT: SO IF THE MONEY WAS  
17 ASSURED, BUT IT WAS ASSURED TO COME IN JANUARY,  
18 WOULD THE INSTITUTION? I THINK PROBABLY.

19 DR. JUELSGAARD: THANK YOU.

20 MR. TOCHER: I'D LIKE TO MAKE A COMMENT  
21 FROM A PROCESS STANDPOINT. WHILE THE SUBCOMMITTEE  
22 MEETING TODAY CAN URGE YOUR COLLEAGUES AT THE BOARD  
23 MEETING WHEN IT DETERMINES ITS BUDGET TO MAKE THIS  
24 ALLOCATION SPECIFIC FOR CERTAIN AWARDS, IT CANNOT  
25 BE, TO THE EARLIER COMMENT, GUARANTEED THAT THAT

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1 WOULD TAKE PLACE. SO I JUST WANT TO MAKE SURE THAT  
2 WE DON'T OVERASSURE SOMETHING THAT IS STILL UP TO  
3 THE VOTE OF THE BOARD WHEN IT MAKES BUDGETARY  
4 ALLOCATIONS.

5 DR. JUELSGAARD: HAVING SAID THAT, SCOTT,  
6 I THINK THE NUMBER OF VOTES THAT ARE BEING COUNTED  
7 TODAY, IF EVERYBODY WERE TO AGREE TO WHAT WE'RE  
8 SAYING RIGHT NOW, IF THE VOTES STAYED THE SAME AS  
9 THEY ARE TODAY, IT WOULD BE DE FACTO APPROVED OR  
10 EVEN DE JURE APPROVED.

11 MR. TOCHER: I UNDERSTAND. I JUST DIDN'T  
12 WANT PEOPLE TO MISCONSTRUE THE WORD "GUARANTEE" IN  
13 ANY SORT OF LEGAL SENSE THAT THIS WAS BINDING ON A  
14 LATER ACTION OF THE BOARD.

15 DR. MARTIN: I DID NOT UNDERSTAND THAT.  
16 WOULD YOU REPEAT THAT CONSTRAINT?

17 MR. TOCHER: SURE. WHAT I WAS POINTING  
18 OUT IS THERE WAS A QUESTION RAISED ABOUT WHETHER OR  
19 NOT THE SUBCOMMITTEE COULD GUARANTEE THAT AT A  
20 FUTURE MEETING THE ADDITIONAL FUNDS WOULD BE  
21 ALLOCATED FOR ADDITIONAL AWARDS BEYOND WHAT IS  
22 BUDGETED FOR TODAY. AND I JUST WANTED TO EMPHASIZE  
23 THE LEGAL POINT, THAT A VOTE, WHILE ALL OF THE  
24 STATEMENTS THAT DR. JUELSGAARD MADE ARE TRUE, IS NOT  
25 LEGALLY BINDING ON THE BOARD WHEN IT NEEDS TO MAKE A

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1 FURTHER BUDGET DECISION, THAT IT WOULD STILL BE UP  
2 TO THE FULL BOARD'S DISCRETION.

3 DR. MARTIN: THANK YOU. I THINK THAT'S AN  
4 IMPORTANT CONSIDERATION.

5 I HATE TO PROPOSE THIS, BUT I WOULD SPLIT  
6 THE BUCKET, NOT SPLITTING BABIES ANYMORE, BUT SPLIT  
7 THE BUCKET, AND I WOULD FUND HALF OF, EACH OF THESE  
8 HALF, PERIOD, AND ENCOURAGE THEM, THE GRANTEES, TO  
9 FIND FUNDING FOR THE OTHER HALF ELSEWHERE AND/OR  
10 REAPPLY NEXT YEAR. I THINK THAT'S A CLEAR PROCESS.  
11 CERTAINLY CIRM IS NOT THE ONLY SOURCE OF FUNDING FOR  
12 THESE IMPORTANT PROJECTS, AND MATCHING FUNDS FROM  
13 INSTITUTIONS ARE CERTAINLY ONE OF THE OTHER  
14 OPPORTUNITIES.

15 CHAIRMAN THOMAS: DAVE, I RESPECTFULLY,  
16 IF WE'RE GOING TO BE SPLITTING ANYTHING, I'D RATHER  
17 FUND HALF NOW AND HALF AT THE BEGINNING AND NOT  
18 LEAVE THE APPLICANTS HANGING TO EITHER WHERE THEY'RE  
19 GOING TO GET THE BALANCE OR HAVING TO WAIT TILL NEXT  
20 JUNE TO REAPPLY. THANK YOU.

21 MR. TOCHER: JUST A SUGGESTION. I'M TOLD  
22 WE'RE ON THE VERGE OF LOSING QUORUM. ONE THING --  
23 ONE OTHER OPTION TO CONSIDER IS WE TRY TO DISPOSE OF  
24 WHAT YOU CAN WITHIN THE BUDGET TODAY. THE NEXT FULL  
25 BOARD MEETING IS ACTUALLY SCHEDULED FOR OCTOBER.

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1 ONE COULD AGENDIZE A BUDGET AUGMENTATION AND NOT  
2 WAIT UNTIL JANUARY FOR THE BROADER DISCUSSION. YOU  
3 WOULD HAVE TO KIND OF ADVANCE THAT DISCUSSION. I  
4 DON'T PROPOSE TO PRESUME THAT THIS HAS BEEN VETTED  
5 INTERNALLY, BUT THAT IS AT LEAST A MORE EXPEDITIOUS  
6 OPTION THAN WAITING TILL DECEMBER OR JANUARY.

7 MR. SHEEHY: WHAT ABOUT IF -- WE HAVE 1.7  
8 MILLION ROUGHLY LEFT. WHY DON'T WE SPLIT THAT  
9 BETWEEN THE TWO PROJECTS AND COMMIT TO AGENDIZING  
10 THE REMAINDER AT THE NEXT BOARD MEETING?

11 MR. TOCHER: THE ONLY QUESTION THEN IS,  
12 JUST TO PLAY IT OUT, IF FOR SOME REASON IT DIDN'T GO  
13 FORWARD, THAT THE ADDITIONAL FUNDS WEREN'T APPROVED  
14 ON THESE APPLICATIONS, YOU WOULD ESSENTIALLY HAVE  
15 STARTED AN APPLICATION AND THEN HAD TO STOP IT IF  
16 THEY WERE UNABLE TO MAKE UP THE SUPPLEMENT FUNDING.  
17 SO YOU MIGHT WANT TO MAKE IT CONTINGENT ON THEM  
18 BEING ABLE TO TAKE UP THAT FUNDING IN THE EVENT THAT  
19 IT WASN'T ULTIMATELY REACHED.

20 DR. JUELSGAARD: I'D ACTUALLY DO IT  
21 DIFFERENTLY. I WOULD DO IT EXACTLY AS JEFF  
22 SUGGESTED. NOW IT'S THEN UP TO THE ACADEMIC  
23 INSTITUTIONS OR WHOEVER IT IS THAT'S RUNNING THIS  
24 WHETHER THEY WANT TO ACCEPT THAT AMOUNT OF MONEY AND  
25 PROCEED FORWARD UNDERSTANDING THERE'S SOME RISK.

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1     ALTHOUGH I PERCEIVE THAT RISK AS MINIMAL, THERE  
2     WOULD BE SOME RISK THAT THEY WOULDN'T GET ADDITIONAL  
3     FUNDING.

4             SO IF I WERE THEM, I'D EITHER START AND  
5     TAKE A CHANCE THE ADDITIONAL FUNDING WILL SHOW UP,  
6     OR I WOULD SIMPLY WAIT TO SEE IF THE ADDITIONAL  
7     FUNDING SHOWS UP. THOSE WOULD BE THE TWO ROADS I  
8     WOULD GO DOWN, BUT I WOULD DO THIS NOW, AND THEN  
9     WE'LL TAKE THE NEXT STEP IN OCTOBER.

10            MR. SHEEHY: THAT MAKES A LOT OF SENSE.

11            MR. TOCHER: SO THE MOTION WOULD BE TO  
12     FUND EACH OF THOSE TWO PROJECTS HALF OF THEIR  
13     BUDGET?

14            MR. SHEEHY: NO.

15            MR. TOCHER: ONE-HALF OF ALL THE REMAINING  
16     DOLLARS.

17            MR. SHEEHY: SPLITTING THE REMAINING  
18     DOLLARS BETWEEN THE TWO APPLICATIONS. SO THE MOTION  
19     IS TO SPLIT THE REMAINING ONE MILLION 730 BETWEEN  
20     THE TWO APPLICATIONS, TO APPROVE THE TWO  
21     APPLICATIONS, TO SUBMIT -- TO SPLIT THE REMAINING  
22     MONEY THAT'S LEFT, THE 1.730, BETWEEN THOSE TWO  
23     APPLICATIONS AND THEN TO BRING THE REMAINDER OF EACH  
24     APPLICATION TO THE BOARD FOR ADDITIONAL FUNDING AT  
25     THE NEXT BOARD MEETING TO COMPLETE THE FUNDING

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REQUEST.

MR. ROWLETT: THAT'S CLEAR AND UNDERSTOOD.

THANK YOU, JEFF.

CHAIRMAN THOMAS: SECOND.

MR. SHEEHY: WE HAVE A SECOND ON THAT.

MR. ROWLETT: I WILL CERTAINLY MAKE A MOTION.

MR. SHEEHY: DO WE HAVE ANY ADDITIONAL COMMENT BY MEMBERS OF THE BOARD? DO WE HAVE ANY ADDITIONAL PUBLIC COMMENT? CAN WE CALL THE ROLL.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: YES.

MS. BONNEVILLE: DAVE MARTIN.

DR. MARTIN: YES.

MS. BONNEVILLE: LAUREN MILLER.

MS. MILLER: YES.

MS. BONNEVILLE: FRANCISCO PRIETO.

DR. PRIETO: AYE.

MS. BONNEVILLE: ROBERT QUINT.

DR. QUINT: YES.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: YES.

MS. BONNEVILLE: JEFF SHEEHY.

MR. SHEEHY: YES.

MS. BONNEVILLE: OS STEWARD.

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1 DR. STEWARD: YES.

2 MS. BONNEVILLE: JONATHAN THOMAS.

3 CHAIRMAN THOMAS: YES.

4 MS. BONNEVILLE: ART TORRES.

5 MR. TORRES: AYE.

6 MS. BONNEVILLE: DIANE WINOKUR.

7 MS. WINOKUR: YES.

8 MS. BONNEVILLE: THE MOTION CARRIES.

9 CHAIRMAN THOMAS: GREAT. THANK YOU.

10 MR. TOCHER: THE REMAINING APPLICATIONS,  
11 YOUR MOTION IS WITH RESPECT TO THE TWO THAT YOU  
12 SPOKE OF.

13 CHAIRMAN THOMAS: WHY DON'T WE CONTINUE  
14 THE REMAINING APPLICATIONS FOR THE NEXT APPLICATION  
15 REVIEW SUBCOMMITTEE? THAT HAPPENS AFTER THE BOARD,  
16 AND WE CAN HAVE A DISCUSSION WITH THE BOARD WHETHER  
17 OR NOT TO FUND THOSE APPLICATIONS OR NOT.

18 MS. BONNEVILLE: YOU WANT TO KEEP THESE  
19 OPEN UNTIL AUGUST OR UNTIL AFTER OCTOBER?

20 MR. SHEEHY: AFTER THE OCTOBER MEETING.  
21 NOW THAT I'VE HAD EXPERIENCE ON OTHER BODIES, SOME  
22 OF THESE CONTINUED THINGS, A NOVEL THING. WE DIDN'T  
23 REALLY REACH A CONSENSUS ON WHAT TO DO WITH THE  
24 OTHER APPLICATIONS AND LET'S HEAR WHAT THE REST OF  
25 THE BOARD BECAUSE THAT WAS A LEGITIMATE AND

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1 INTERESTING IDEA. WE'RE GOING TO ASK THEM TO FUND  
2 THE REMAINDER, WHICH WILL BE, WHAT, ABOUT 600,000  
3 MAYBE OR 700,000 UNLESS MY MATH IS OFF FOR THOSE  
4 TWO, AND THEN WE CAN DISCUSS WHETHER THERE'S AN  
5 INTEREST IN FUNDING THE REST OF THESE -- PUTTING  
6 MONEY IN FOR THE REST OF THESE APPLICATIONS. WE  
7 DON'T HAVE TO DISPOSE OF THEM AT THIS TIME. THEY'RE  
8 APPLICATIONS WITH MERIT, BUT WE GET THE SEVEN THAT  
9 WE WANTED TO GET MOVING FORWARD. WE SHOULD BE ABLE  
10 TO GET THE REMAINDER OF THAT FUNDING AT THE NEXT  
11 BOARD MEETING, AND THEN WE CAN HAVE A DISCUSSION  
12 THERE ON WHAT THEY WANT TO DO ABOUT THE DISCOVERY  
13 ROUND FOR NEXT YEAR. AND WE CAN LET MORE MEMBERS OF  
14 THE BOARD PARTICIPATE IN THAT DECISION.

15 CHAIRMAN THOMAS: I THINK THAT'S AN  
16 EXCELLENT IDEA, MR. SHEEHY.

17 MR. SHEEHY: UNLESS SOMEBODY HAS ANY  
18 COMMENTS.

19 DR. STEWARD: I HAVE A QUESTION. AND THIS  
20 IS FOR SCOTT. SO DOES THAT MEAN THAT THE NEXT  
21 DISCUSSION OF THIS WOULD BE THE APPLICATION REVIEW  
22 SUBCOMMITTEE OR THE WHOLE BOARD, OR HOW DOES THAT  
23 WORK?

24 MR. TOCHER: THE FIRST DISCUSSION WOULD BE  
25 THE WHOLE BOARD, WHICH WOULD DETERMINE WHETHER OR



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1 NOT TO ALLOCATE ADDITIONAL FUNDS. ASSUMING THAT WAS  
2 ANSWERED IN THE AFFIRMATIVE, WE WOULD THEN MEET AS  
3 THE APPLICATION REVIEW SUBCOMMITTEE WHO WOULD MAKE  
4 DETERMINATION REGARDING THE REMAINDER OF BUDGETS OF  
5 THESE TWO APPLICATIONS THAT ARE PARTIALLY FUNDED AND  
6 THEN TO DISPOSE OF ONE WAY OR THE OTHER WITH THE  
7 REMAINING APPLICATIONS. LIKE TODAY, IT WOULD BE A  
8 JOINT MEETING. DOES THAT ANSWER YOUR QUESTION, OS?

9 DR. STEWARD: YES, IT DID. THANK YOU VERY  
10 MUCH.

11 MR. SHEEHY: OKAY. IS THERE ADDITIONAL  
12 PUBLIC COMMENT AT ANY OF THE SITES?

13 DR. STEWARD: JEFF, THIS IS OS AGAIN. AND  
14 I WONDER -- I THINK THAT THE MOTION AS IT CURRENTLY  
15 STANDS IS A TWO-PART MOTION, WHICH IS TO FUND THESE  
16 TWO POTENTIALLY AT HALF AND THEN TO DEFER THE REST;  
17 IS THAT CORRECT?

18 MR. SHEEHY: THE MOTION IS TO FUND THESE  
19 TWO APPLICATIONS WITH THE 1.7 MILLION, WHICH IS  
20 ABOUT TWO-THIRDS. THERE'S A REMAINDER OF ABOUT ONE  
21 MILLION THAT WE WILL BE REQUESTING THE BOARD TO FUND  
22 TO FINISH COMPLETING THE FUNDING FOR THE AWARDS. AT  
23 THAT SAME BOARD, WE HAVE CONTINUED THE REMAINDER OF  
24 THE APPLICATIONS, AND WE WILL HEAR AT THE BOARD, WE  
25 WILL AGENDA THE BOARD THE DISCUSSION OF WHETHER THEY

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1 WANT TO PUT IN ADDITIONAL MONEY FOR THESE  
2 APPLICATIONS BECAUSE IT WILL HAVE AN IMPACT ON NEXT  
3 YEAR'S BUDGET. AND THAT SHOULD BE A FULL BOARD  
4 DISCUSSION. IF THEY DON'T WANT TO PUT ANY  
5 ADDITIONAL MONEY IN, THEN THAT'S THAT. IF THEY DO  
6 DECIDE TO PUT IN ADDITIONAL MONEY, THEN WE CAN HAVE  
7 A VOTE AT THE APPLICATION REVIEW SUBCOMMITTEE.  
8 THESE REMAINING AWARDS ARE NOT FUNDED AS OF THIS  
9 MEETING. THEY'RE CONTINUED. DOES THAT MAKE SENSE?

10 DR. STEWARD: IT DOES MAKE SENSE, BUT I  
11 WANT TO JUST SAY THAT I WILL BE IN FAVOR OF YES FOR  
12 FUNDING THESE TWO AT HALF. I'M NOT IN FAVOR OF THE  
13 CONTINUATION SIMPLY BECAUSE, AGAIN, I RAISED A POINT  
14 THAT I THINK WE NEED TO START IMPOSING DISCIPLINE ON  
15 OURSELVES AND MAKING THE HARD DECISIONS THAT WE KNOW  
16 ARE GOING TO NEED TO BE MADE. SO I WOULD NOT VOTE  
17 FOR THE SECOND PART OF THAT MOTION IF IT'S PART OF  
18 THE MOTION. IF IT'S NOT A VOTE INVOLVED, THAT'S  
19 FINE. THANK YOU.

20 MR. SHEEHY: THERE'S NOT A VOTE INVOLVED.  
21 IT'S ACTUALLY AT THE DISCRETION OF CHAIRMAN THOMAS  
22 TO AGENDA THE ITEM, WHICH I THINK HE INTENDS TO  
23 AGENDA.

24 CHAIRMAN THOMAS: CORRECT.

25 DR. CHANG: HI. I'M ALAN CHANG. I'M ONE

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1 OF THE PHYSICIAN SCIENTISTS FROM STANFORD. I'M HERE  
2 TO ADVOCATE FOR MY GRANT 11199. IT'S A VERY COMMON  
3 DISEASE. IT AFFECTS ABOUT HALF A BILLION WORLDWIDE  
4 AND TWO MILLION CALIFORNIANS, AND THERE'S VERY  
5 LITTLE FUNDING FOR IT. I WAS PREVIOUSLY FUNDED BY  
6 CIRM TO BASICALLY ESTABLISH THE MODEL AND THE  
7 PRELIMINARY DATA TO ALLOW ME TO APPLY WHAT I PUT  
8 TOGETHER RIGHT NOW.

9 IT'S STILL A VERY COMMON PROBLEM THAT  
10 AFFECTS US DAY-TO-DAY COMMUNICATION TO OUR EMOTIONAL  
11 STATUS, SOCIAL STATUS. AND IN KIDS, THEY CANNOT  
12 HAVE SPEECH IF THEY DON'T HAVE HEARING. ACTUALLY  
13 HAD A MOM WHO HAD BEEN WAITING FOR TREATMENT ON THE  
14 PHONE, AND HER CHILD ACTUALLY LOST HEARING, THAT WE  
15 HAVE TO PUT A COCHLEAR IMPLANT IN JUST A FEW WEEKS  
16 AGO. RIGHT NOW THERE IS NO BIOLOGICAL TREATMENT.  
17 YOU CAN ONLY USE HEARING AIDS. IF THERE'S NO  
18 HEARING, THEN WE HAVE TO USE COCHLEAR IMPLANTS.

19 I THINK WE HAVE A VERY NICE AND EXCITING  
20 PROGRAM THAT I THINK WILL MAKE A BIG DIFFERENCE TO  
21 OUR PATIENTS IN CALIFORNIA. SO I HOPE I CAN GET  
22 YOUR SUPPORT ON OUR PROGRAM.

23 MS. CHEUNG: WE HAVE SOMEONE ON THE PHONE.  
24 NEAL, WE'RE PUTTING YOU THROUGH IF YOU'RE STILL  
25 THERE.

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1 DR. SIEGEL: I AM. THIS IS NEAL SIEGEL.  
2 I'M AT UCS. I'M ADVOCATING FOR THE GRANT 11183, A  
3 SCREEN FOR DRUGS TO PROTECT AGAINST  
4 CHEMOTHERAPY-INDUCED HEARING LOSS. LET ME START BY  
5 EXPLAINING THE SCOPE OF THE PROBLEM.

6 THIS IS PLATIN-BASED CHEMOTHERAPY IS THE  
7 TREATMENT OF CHOICE FOR MANY PEDIATRIC CANCERS, SUCH  
8 AS NEUROBLASTOMA, AND IT HAS A HIGH SUCCESS RATE,  
9 GREATER THAN 80 PERCENT. HOWEVER, IT ALSO HAS  
10 SEVERE SIDE EFFECTS INCLUDING PROFOUND HEARING LOSS  
11 IN OVER 60 PERCENT OF THOSE SURVIVORS. WHILE ADULT  
12 CANCER SURVIVORS ALSO EXPERIENCE SIGNIFICANT HEARING  
13 LOSS, IT IS PARTICULARLY A PROBLEM IN YOUNG  
14 CHILDREN, MANY OF WHOM HAVE NOT LEARNED TO SPEAK.  
15 THUS, LEADING TO DELAYS IN LANGUAGE ACQUISITION AND  
16 COGNITIVE DEVELOPMENT EVEN WITH INTENSIVE THERAPY.

17 LET ME ALSO SAY THAT TO DATE CIRM HAS  
18 PROVIDED THREE HEARING LOSS GRANTS IN ITS HISTORY  
19 WHILE FUNDING 25 OR MORE GRANTS RELATED TO  
20 BLINDNESS. SO IT'S AN UNDERSERVED GROUP. DEAFNESS  
21 IS CAUSED BY THE HYPERSENSITIVITY OF THE SENSORY  
22 HAIR CELLS OF THE INNER EAR, TO ENVIRONMENTAL STRESS  
23 LIKE THESE PLATINATED CHEMOTHERAPY AGENTS; AND WHEN  
24 HAIR CELLS DIE, THEY DO NOT REGENERATE AS DR. CHANG  
25 JUST MENTIONED. CURRENTLY THERE ARE NO PROTECTIVE

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1 DRUGS AVAILABLE TO AMELIORATE THE SIDE EFFECTS THAT  
2 ACCOMPANY THIS LIFESAVING TREATMENT. AN ADJUVANT  
3 DRUG THERAPY THAT CAN BE ADMINISTERED ALONG WITH  
4 CISPLATIN TO PROTECT HEARING WOULD BE VERY WELCOME.

5 HAIR CELLS HAVE LONG BEEN KNOWN TO BE  
6 HYPERSENSITIVE TO THESE CHEMOTHERAPY AGENTS, AND WE  
7 HAVE RECENTLY BEEN SUCCESSFUL IN USING A DIRECT  
8 LINEAGE REPROGRAMMING APPROACH TO GENERATE LARGE  
9 NUMBERS OF THESE HAIR CELLS FROM MOUSE FIBROBLASTS  
10 USING A COCKTAIL OF TRANSCRIPTION FACTORS AND IN  
11 PILOT STUDIES HAVE USED THIS SAME COCKTAIL OF  
12 TRANSCRIPTION FACTORS TO INDUCE HUMAN IPS CELLS  
13 DERIVED FIBROBLASTS TO BECOME HAIR CELL-LIKE. WITH  
14 THIS PROCESS, THESE INDUCED HAIR CELLS HAVE  
15 PROPERTIES EXTREMELY SIMILAR TO NORMAL HAIR CELLS,  
16 INCLUDING THE HYPERSENSITIVITY TO CHEMOTHERAPY  
17 AGENTS.

18 SO OUR PROPOSAL TO CIRM IS TO PRODUCE A  
19 HIGH THROUGHPUT SCREEN TO DISCOVER DRUGS THAT ARE  
20 ABLE TO PROTECT HAIR CELLS DURING LIFESAVING  
21 CHEMOTHERAPY. AS I MENTIONED, CIRM HAS PREVIOUSLY  
22 FUNDED ONLY THREE HEARING LOSS GRANTS, AND SO I  
23 BELIEVE THIS IS PARTLY A REFLECTION OF THE PAUCITY  
24 OF STEM CELL APPROACHES FOR STUDYING HEARING LOSS IN  
25 HUMANS. OUR NEW DIRECT LINEAGE REPROGRAMMING

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1 APPROACH USING HUMAN INDUCED HAIR CELLS FOR DRUG  
2 DISCOVERY PROMISES TO OVERCOME THESE PROBLEMS AND I  
3 THINK DESERVE CIRM'S FURTHER CONSIDERATION.

4 I'D ALSO LIKE TO SAY THAT, WHILE I REALIZE  
5 THE DIFFICULTY THAT THE BOARD HAS AND THE DISCIPLINE  
6 THAT DR. STEWARD SUGGESTS IS NEEDED FOR MAKING THESE  
7 DECISIONS, THE PROCESS OF MAKING THOSE DECISIONS IS  
8 A LITTLE FRUSTRATING LISTENING TO THIS OVER THE  
9 PHONE GIVEN THAT ALL DECISIONS HAVE ALMOST BEEN MADE  
10 BEFORE YOU'VE HEARD THESE PUBLIC COMMENTS. THANK  
11 YOU.

12 MR. SHEEHY: THANK YOU.

13 MS. CHEUNG: ONE MORE FROM THE PREVIOUS  
14 GRANT. LENA, WE'RE GOING TO PUT YOU THROUGH  
15 SHORTLY. ONE SECOND. WE'RE JUST PUTTING YOU  
16 THROUGH NOW. CAN YOU HIT STAR ONE ON YOUR PHONE  
17 PLEASE, LENA? YOU CAN GO AHEAD AND SPEAK, LENA.

18 LENA: MY NAME IS LENA, AND I AM A MOTHER  
19 OF A CHILD WITH HEARING LOSS. HER HEARING LOSS IS  
20 GENETIC. AND, YOU KNOW, IT WAS LIFECHANGING WHEN WE  
21 DISCOVERED THAT OUR DAUGHTER HAD A HEARING LOSS  
22 ESPECIALLY BECAUSE WE START LOOKING FOR  
23 ALTERNATIVES. AND THERE'S NO CURE FOR THAT. AND,  
24 YOU KNOW, THE ONLY OPTION THAT WE HAVE WAS HEARING  
25 AIDS. AND RIGHT NOW WE'RE LOOKING AT -- SHE HAS A

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1 COCHLEAR IMPLANT RECENTLY.

2 IT'S BEEN REALLY TOUGH. IT'S A TOUGH  
3 JOURNEY JUST TO SEE A CHILD. IT STARTS WITH A BABY,  
4 AND THEN IT'S GOING TO HAVE THIS FOR HER WHOLE LIFE.  
5 AND HER HEARING LOSS IS GENETIC. SO IT'S TOUGH FOR  
6 PARENTS THAT HAVE MORE KIDS, MORE THAN ONE KID WITH  
7 HEARING LOSS. AND WE REALLY HOPE THAT IN THE FUTURE  
8 WHEN MY DAUGHTER DECIDES TO HAVE KIDS, THERE WILL BE  
9 A CURE FOR HER FAMILY AND NOT HAVE THE WORRY AS WE  
10 DID WITH HER WHEN SHE WAS A BABY AND ALL OF THAT.

11 AND I WOULD LIKE TO SHARE THAT IT'S BEEN  
12 TOUGH NOT ONLY FOR THE CHILD, BUT ALSO FOR THE  
13 FAMILY. THIS IS A JOURNEY THAT IS TOUGH FOR THE  
14 WHOLE FAMILY, AND IT AFFECTS THE WHOLE FAMILY.  
15 WE'RE, LIKE, IN THIS TOGETHER, AND IT'S REALLY HARD.  
16 I WILL LOVE TO SEE THERE'S FUNDING FOR THIS JUST TO  
17 FIND A CURE BECAUSE, LIKE US, IT IS GENETIC, BUT  
18 THERE'S SO MANY KIDS OUT THERE WITH A HEARING LOSS,  
19 AND ALL OF THEM ARE DIFFERENT REASONS FOR THEIR  
20 HEARING LOSS. AS A PARENT, IT IS HARD. AND IT IS  
21 AMAZING UNTIL YOU ARE IN OUR SHOES TO SEE THAT  
22 CALIFORNIA HAS SO MANY KIDS WITH HEARING LOSS. AND  
23 IT WILL BE REALLY, REALLY WONDERFUL TO FIND A CURE  
24 FOR THESE KIDS. THESE KIDS ARE THE FUTURE OF THIS  
25 COUNTRY, AND WE JUST WANT THE BEST FOR THEM.

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1 MR. SHEEHY: THANK YOU. SO NO ADDITIONAL  
2 PUBLIC COMMENT OR BUSINESS BEFORE THE APPLICATION  
3 REVIEW SUBCOMMITTEE? THAT CLOSES THIS APPLICATION  
4 REVIEW SUBCOMMITTEE, AND IT IS THEN NOW BACK TO YOU,  
5 CHAIRMAN THOMAS.

6 CHAIRMAN THOMAS: THANK YOU, MR. SHEEHY.  
7 IS THERE ANY PUBLIC COMMENT ON ANY TOPIC INCLUDING  
8 ANYTHING ABOVE AND BEYOND THAT RELEVANT TO TODAY'S  
9 APPLICATION REVIEW SUBCOMMITTEE MEETING? HEARING  
10 NONE, THAT CONCLUDES OUR AGENDA, AND THANK YOU,  
11 EVERYBODY, FOR YOUR PARTICIPATION AS ALWAYS. WE  
12 STAND ADJOURNED.

13 (THE MEETING WAS THEN ADJOURNED AT  
14 1:03 PM.)

15  
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17  
18  
19  
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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 AND THE APPLICATION REVIEW SUBCOMMITTEE OF THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE IN THE MATTER OF ITS REGULAR MEETING HELD ON JULY 19, 2018, WAS HELD AS HEREIN APPEARS AND THAT THIS IS THE ORIGINAL TRANSCRIPT THEREOF AND THAT THE STATEMENTS THAT APPEAR IN THIS TRANSCRIPT WERE REPORTED STENOGRAPHICALLY BY ME AND TRANSCRIBED BY ME. I ALSO CERTIFY THAT THIS TRANSCRIPT IS A TRUE AND ACCURATE RECORD OF THE PROCEEDING.

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