BEFORE THE

INDEPENDENT CITIZENS' OVERSIGHT COMMITTEE
TO THE
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE
ORGANIZED PURSUANT TO THE
CALIFORNIA STEM CELL RESEARCH AND CURES ACT

REGULAR MEETING

LOCATION:	MARRIOTT LA JOLLA 4240 LA JOLLA VILLAGE DRIVE LA JOLLA, CALIFORNIA
DATE:	AUGUST 28, 2013 9 A.M.
REPORTER:	BETH C. DRAIN, CSR CSR. NO. 7152
BRS FILE NO.:	92761

I N D E X

I N D E X (CONT'D.)

SAN DIEGO, CALIFORNIA; WEDNESDAY, AUGUST 28, 2013

9 A.M.

CHAIRMAN THOMAS: LADIES AND GENTLEMEN, WE'RE WAITING FOR A VERY IMPORTANT ARRIVAL OF ONE ADDITIONAL PERSON, SO WE WILL START AS SOON AS HE GETS HERE, WHICH SHOULD BE PRESENTLY. SO BEAR WITH US FOR A SECOND. THANK YOU.

MEMBERS OF THE BOARD, I'M TOLD THAT IN ORDER TO USE TODAY'S MICROPHONE, YOU HAVE TO KEEP YOUR FINGER ON THE ON BUTTON SO THAT THE MIC STAYS ON. A LITTLE LOGISTICALLY DIFFICULT, BUT YOUR FINGER WILL GET A GOOD WORKOUT.

SO WHILE WE'RE WAITING FOR OUR GUEST, WE CAN GET THROUGH A COUPLE OF EARLY MATTERS HERE. SO I'D LIKE TO CALL THE MEETING OF THE ICOC TO ORDER FROM THE LA JOLLA MARRIOTT IN SAN DIEGO ON THIS AUGUST 28TH. WELCOME EVERYBODY. IT'S A PLEASURE TO SEE YOU ALL HERE AS USUAL.

LET'S GO FIRST TO MARIA TO LEAD US IN THE PLEDGE OF ALLEGIANCE.

(THE PLEDGE OF ALLEGIANCE.)

CHAIRMAN THOMAS: MARIA, PLEASE CALL THE ROLL.

MS. BONNEVILLE: SUE BRYANT.

DR. BRYANT: HERE.
MS. BONNEVILLE: KEN BURTIS.
DR. BURTIS: PRESENT.
MS. BONNEVILLE: KRISTINA VUORI.
DR. VUORI: HERE.
MS. BONNEVILLE: ANNE-MARIE DULIEGE.
DR. DULIEGE: HERE.
MS. BONNEVILLE: MARCY FEIT. LEON FINE.
DR. FINE: HERE.
MS. BONNEVILLE: ELIZABETH FINI.
DR. FINI: HERE.
MS. BONNEVILLE: MICHAEL FRIEDMAN.
DR. FRIEDMAN: HERE.
MS. BONNEVILLE: MICHAEL GOLDBERG.
MR. GOLDBERG: HERE.
MS. BONNEVILLE: SAM HAWGOOD.
DR. HAWGOOD: HERE.
MS. BONNEVILLE: STEPHEN JUELSGAARD.
MR. JUELSGAARD: HERE.
MS. BONNEVILLE: SHERRY LANSING. BERT LUBIN.
DR. LUBIN: HERE.
MS. BONNEVILLE: LLOYD MINOR.
DR. MINOR: HERE.
MS. BONNEVILLE: KIRK PETERSON.

DR. PETERSON: HERE.

MS. BONNEVILLE: FRANCISCO PRIETO.

DR. PRIETO: HERE.

MS. BONNEVILLE: ROBERT QUINT.

DR. QUINT: HERE.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: HERE.

MS. BONNEVILLE: JOAN SAMUELSON.

MS. SAMUELSON: HERE.

MS. BONNEVILLE: JEFF SHEEHY.

MR. SHEEHY: HERE.

MS. BONNEVILLE: OSWALD STEWARD. JONATHAN THOMAS.

CHAIRMAN THOMAS: HERE.

MS. BONNEVILLE: ART TORRES.

MR. TORRES: HERE.

MS. BONNEVILLE: EUGENE WASHINGTON. DONNA WESTON.

DR. WESTON: HERE.

MS. BONNEVILLE: DIANE WINOKUR.

MS. WINOKUR: HERE.

CHAIRMAN THOMAS: THANK YOU. ALAN, BY THE WAY, WE'RE GOING TO SAVE YOUR REPORT FOR THE TAIL END HERE IF THAT'S OKAY BECAUSE WE NEED TO GET THROUGH SOME CERTAIN ITEMS BEFORE WE LOSE A MEMBER

OR TWO. SO WE'LL DO THE PRESIDENT'S REPORT AS THE GRAND WRAP-UP TO THE PROCEEDINGS.

WHILE WE'RE WAITING, PERHAPS WE CAN PROCEED TO ITEM NO. 8, WHICH IS CONSIDERATION OF FINAL ADOPTION OF AMENDMENTS TO CIRM INTELLECTUAL PROPERTY REGULATIONS. STEVE, WOULD YOU JUST LIKE TO COMMENT A BIT ON THE IP AND INDUSTRY SUBCOMMITTEE MEETING AND THEN INTRODUCE ELONA.

MR. JUELSGAARD: SO LAST EVENING WE HELD A MEETING OF THE IP AND INDUSTRY SUBCOMMITTEE TO REVIEW CERTAIN PROPOSED AMENDMENTS TO THE REGULATIONS IN THE INTELLECTUAL PROPERTY AREA THAT STAFF HAD BEEN WORKING ON. AND I WON'T GO INTO THEM IN DETAIL BECAUSE ELONA IS GOING TO DO THAT IN A SECOND. BUT THE IP AND INDUSTRY SUBCOMMITTEE RECOMMENDED TO THE ICOC THAT THEY ADOPT THESE CHANGES. AND SO WITH THAT, I'LL ASK ELONA TO RECOUNT WHAT THOSE PROPOSED CHANGES ARE.

MS. BAUM: GOOD MORNING AND THANK YOU VERY MUCH FOR CONSIDERING THIS ITEM BEFORE YOU. BY WAY OF BACKGROUND, I JUST WANT TO REMIND EVERYBODY THAT IN OCTOBER OF 2012 A NUMBER OF THE AMENDMENTS THAT YOU SEE BEFORE YOU TODAY, ESPECIALLY THOSE THAT ARE MARKED IN RED IN THE DOCUMENTATION YOU HAVE, HAVE BEEN REVIEWED AND EXTENSIVELY CONSIDERED AND

DELIBERATED AND ULTIMATELY APPROVED BY THE BOARD. AND AS A RESULT, WE POSTED THOSE AMENDMENTS AND SOME ADDITIONAL AMENDMENTS THAT ARE HIGHLIGHTED IN YELLOW IN THE DOCUMENTATION YOU HAVE FOR PUBLIC COMMENT, OPENED UP A RULEMAKING PROCEEDING.

AND WE DID NOT RECEIVE ANY FORMAL WRITTEN COMMENTS, AND NOW WE ARE COMING BEFORE THE BOARD TO, ONE, REQUEST THAT YOU APPROVE ALL OF THE PROPOSED CHANGES THAT ARE IN RED, WHICH YOU ALREADY APPROVED SO I WON'T SPEND MUCH TIME TALKING ABOUT, AND IN ADDITION, THESE HIGHLIGHTED PROVISIONS AND IN PARTICULAR IF THERE'S AN OPTION, AND I'LL GET INTO IT, OPTION A. SO THAT'S WHAT WE'RE ASKING TODAY AND I'LL GO THROUGH THEM AS WE PROCEED.

THERE'S A COUPLE NEW DEFINITIONS THAT WE MADE SOME CHANGES TO, AND WE MADE SOME CHANGES TO LICENSING REVENUE PROVISIONS AND REPORTING OF LICENSING AGREEMENTS, WHICH I'LL DESCRIBE.

SO I THINK WHAT I SHOULD DO IS FIRST START WITH THE EASIEST ITEM IN MANY RESPECTS, AND THAT IS THE REPORTING OF LICENSING ACTIVITIES. THAT'S SECTION 100602. YOU MIGHT RECALL THAT THE BOARD HAD APPROVED THAT WITHIN 60 DAYS OF EXECUTION OF A LICENSING AGREEMENT THAT A GRANTEE WAS REQUIRED TO PROVIDE US A COPY OF THAT. THAT WAS ALREADY

APPROVED. THAT'S IN RED. BUT ALTHOUGH WE DIDN'T RECEIVE FORMAL WRITTEN COMMENTS TO ADDRESS THAT PROPOSED AMENDMENT, WHAT WE DID RECEIVE IS SOME INFORMAL TELEPHONE DISCUSSIONS WITH THE OFFICE OF THE PRESIDENT OF THE UC. AND THEY PRESENTED SOME CONCERNS AND SOME DIFFICULTIES. SO WHAT WE'VE DONE IS PROVIDED IN HIGHLIGHT SOME VERBIAGE THAT ADDRESSES THOSE CONCERNS, AND ALL ARE IN AGREEMENT WITH THAT NEW VERSION.

SO WHAT WE ARE PROPOSING IS THAT INSTEAD OF 60 DAYS TO PROVIDE A WRITTEN DISCLOSURE OF THE LICENSING AGREEMENT THAT'S ENTERED INTO, IT'S 90 DAYS. GIVES THEM 30 MORE DAYS TO ADDRESS THIS.

AND INSTEAD OF ACTUALLY PROPOSING OR PROVIDING AN EXECUTED COPY OF THE WHOLE AGREEMENT, THEY NEED ONLY PROVIDE US WITH PROVISIONS RELATING TO THE LICENSING REVENUE. AND IN ADDITION, WE FOR MAXIMUM FLEXIBILITY HAVE BUILT IN A PROVISION THAT ALLOWS CIRM AND THE GRANTEE TO DISCUSS OTHER MEANS OF DISCLOSURE. SO INSTEAD OF PERHAPS ACTUALLY PRODUCING A REDACTED COPY OF THE LICENSING AGREEMENT, PERHAPS CIRM MIGHT FEEL IT'S APPROPRIATE TO AGREE TO DELIVERY THROUGH A DATA ROOM.

AND IN ADDITION, WE ADDED SOME CLARIFYING LANGUAGE THAT STATES THAT WE NOT ONLY WANT TO HAVE

INFORMATION REGARDING LICENSING AGREEMENTS AND IN PARTICULAR REVENUE SHARING, BUT WE ALSO WANT TO KNOW WHEN THEY ENTER INTO MTA'S AND COLLABORATION AGREEMENTS. SO THIS IS ALL HIGHLIGHTED IN THE TEXT BEFORE YOU. IT'S A WAY TO ENSURE THAT CIRM HAS MORE PROMPT NOTICE BECAUSE, ALTHOUGH THE GRANTEES ARE REQUIRED TO GIVE US ANNUAL NOTICE BEFORE WE EVEN ISSUE THE 60-DAY AMENDMENT, WE WERE FINDING THAT WE WERE CONSTANTLY QUESTIONED ABOUT WHERE THE STATUS IS IN TERMS OF ENGAGEMENT OF OUTSIDE ENTITIES TO CARRY ON OUR WORK; I.E., LICENSING, ETC. SO WE FELT THAT AT LEAST QUARTERLY REPORTING WOULD HELP US MEET THOSE QUESTIONS.

THEN WE ALSO MADE SOME WHAT I CALL CLEANUP CHANGES TO A FEW DEFINITIONS IN 601, IN PARTICULAR EXCLUSIVE LICENSEE, EXCLUSIVE LICENSE, LICENSE AGREEMENT, AND LICENSING REVENUE. WE MADE SOME ENHANCEMENTS OR CLARIFICATIONS. WE WANTED TO MAKE SURE THAT WE DIDN'T TOO NARROWLY DEFINE THOSE DEFINITIONS. SO WHAT WE DID, AND PARDON ME, BUT I HAVE TO REVERT TO LEGALESE HERE, IS WE INCORPORATED A CONCEPT OF A COVENANT NOT TO SUE FOR INFRINGEMENT INTO THE DEFINITION. IT'S IN ORDER TO ENSURE THAT OUR DEFINITIONS FULLY ENCOMPASS THE DIFFERENT SCENARIOS THAT MAKE SENSE WITHIN THE DEFINITIONS OF

EXCLUSIVE LICENSEE, LICENSE, ETC.

I THINK MORE RELEVANT FOR YOUR CONSIDERATION, BECAUSE IT IS MORE SUBSTANTIVE THAN WHAT I JUST DESCRIBED, IS A CHANGE THAT WE'RE PROPOSING VIS-A-VIS LICENSING REVENUE. YOU MAY RECALL THAT THE BOARD HAD AGREED THAT LICENSING REVENUE WOULDN'T INCLUDE PRECOMMERCIAL CONSIDERATION RECEIVED BY A FOR-PROFIT. THAT WAS IN LINE WITH WHAT WE'VE DONE WITH THE LOAN ADMINISTRATION POLICY. AND AS WE WERE LOOKING AT THE MULTITUDE OF HUNDREDS OF POSSIBLE SCENARIOS WITH MY COLLEAGUES, SCOTT TOCHER, BEN HUANG, WHO I WANT TO THANK FOR HELPING ME WITH THIS, WE REALIZED THAT THERE MIGHT BE A CERTAIN SITUATION WHERE THAT MIGHT BE A LITTLE TOO BROAD. SO WE HAVE A LITTLE NARROWING OF THAT EXCEPTION THAT'S BEEN PROPOSED, AGAIN, IN YELLOW HIGHLIGHT.

WHAT WE WANT TO SAY IS THAT EXCEPTION TO LICENSING REVENUE A PRECOMMERCIAL REVENUE OBTAINED BY FOR-PROFITS ONLY APPLIES IF THE FOR-PROFIT ENTITY HAS EXPENDED OR IS EXPENDING FUNDS TO SUPPORT THE CIRM-FUNDED INVENTION AND TECHNOLOGY.

AND THEN THERE'S A COUPLE OTHER ITEMS THAT ARE ALSO MARKED IN HIGHLIGHT WITHIN THAT DEFINITION, AND THEY'RE HIGHLIGHTED SIMPLY BECAUSE THEY WERE

MOVED FROM A DIFFERENT PART OF THE TEXT. SO THERE'S NO SUBSTANTIVE CHANGE THERE.

IN ADDITION, WE THOUGHT WE WOULD MAKE SOME CHANGES TO NET COMMERCIAL REVENUE. AND IN DOING SO, WE PROVIDED AN OPTION A, AN OPTION B, AND AN OPTION C WITHIN THE DOCUMENTATION YOU HAVE BEFORE YOU. AS IT TURNS OUT, WE HAVE DECIDED THAT WE WANT TO STAY WITH THE STATUS QUO FOR NOW. SO WE'RE RECOMMENDING THE ADOPTION OF OPTION A THAT APPEARS IN THAT PROPOSED AMENDMENT FOR NET COMMERCIAL REVENUE. OPTION A, AS I SAID, IS WHAT HAS BEEN APPROVED CURRENTLY. AND IT'S OUR INTENT TO COME BACK TO THIS BOARD IN A NEW RULEMAKING PROCEDURE TO PROVIDE A MEANS WHERE WE CAN TELL THE REGULATED PUBLIC THROUGH OUR REGULATIONS WHAT THE INTENT IS VIS-A-VIS THE REVENUE STREAMS THAT ARE APPLICABLE TO OUR REVENUE SHARING PROVISIONS; IN OTHER WORDS, REACH THROUGH.

SO A QUESTION CAN SOMETIMES ARISE, FOR INSTANCE, I'M JUST GIVING YOU ONE SCENARIO, IF WE END UP PROVIDING FUNDING THAT CREATES A TOOL, AND THROUGH USE OF THE TOOL YOU DISCOVER A DRUG. DO WE SEEK THE REVENUES FROM THE DRUG, OR DO WE SEEK THE REVENUES FROM OUT LICENSING THE TOOL? THAT'S SOMETHING THAT WILL REQUIRE, I THINK, A LOT MORE DISCOURSE AND WE'LL ADDRESS AT ANOTHER TIME. SO FOR

NOW WE THINK WE SHOULD JUST KEEP WITH WHAT WE HAVE. AND I THINK SOME PEOPLE WOULD WONDER WHAT THE REACH IS, AND WE'LL TRY TO CLEAR THAT UP IN SUBSEQUENT RULEMAKING. OR IT MIGHT BE THAT WE SIMPLY HAVE AN FAQ AND WE ESTABLISH WHAT THE INTENT IS. WE HAVE TO EXPLORE THAT.

AND FINALLY, YOU WILL SEE SOME HIGHLIGHTED TEXT IN THE REVENUE SHARING PROVISION, WHICH IS 600608. AND THAT'S ESSENTIALLY BECAUSE THE TEXT WAS EITHER MOVED, FOR INSTANCE, IT WAS MOVED TO THE LICENSING REVENUE SECTION, OR IT WAS ELIMINATED BECAUSE IT WAS DUPLICATIVE. SO THERE'S NOTHING SUBSTANTIVE IN THAT CHANGE, BUT WE HAD TO HIGHLIGHT IT BECAUSE IT IS A CHANGE.

WITH THAT, I WOULD REQUEST THAT SOMEBODY MOVE TO APPROVE ALL PROPOSED AMENDMENTS AS DESCRIBED AND SPECIFICALLY OPTION A OF THE THREE OPTIONS THAT APPEAR WITHIN NET COMMERCIAL REVENUE. THANK YOU.

CHAIRMAN THOMAS: DO I HEAR A MOTION TO THAT EFFECT?

MR. JUELSGAARD: I'LL MOVE THAT WE ACCEPT THE PROPOSED CHANGES TO THE REGULATIONS, INCLUDING THE OPTION.

CHAIRMAN THOMAS: MOVED BY MR. JUELSGAARD.

DR. MINOR: SECOND.

CHAIRMAN THOMAS: SECONDED BY DEAN MINOR. IS THERE ANY DISCUSSION BY MEMBERS OF THE BOARD?

MR. GOLDBERG: NO, BUT WE HAVE MEMBERS OF THE PUBLIC WHEN YOU'RE READY HERE.

CHAIRMAN THOMAS: ANY COMMENTS BY THOSE ON THE PHONE? THANK YOU. ANY COMMENTS BY MEMBERS OF THE PUBLIC? HEARING NONE, MR. HARRISON, IS IT OKAY TO HAVE A VOICE VOTE ON THIS?

MR. HARRISON: YOU HAVE TO DO A ROLL CALL OF THOSE ON THE PHONE.

CHAIRMAN THOMAS: ROLL CALL INCLUDING THOSE ON THE PHONE?

MR. HARRISON: NO. ROLL CALL JUST OF THOSE ON THE PHONE.

CHAIRMAN THOMAS: ALL THOSE IN THE ROOM HERE IN FAVOR OF THE MOTION PLEASE SAY AYE. OPPOSED? ROLL CALL ON THE PHONE.

MS. BONNEVILLE: MICHAEL FRIEDMAN.

DR. FRIEDMAN: YES.

MS. BONNEVILLE: MICHAEL GOLDBERG.

MR. GOLDBERG: YES.

MS. BONNEVILLE: LEON FINE.

DR. FINE: YES.

CHAIRMAN THOMAS: THANK YOU, GENTLEMEN. THE MOTION PASSES.

WE'RE NOW GOING TO GO TO THE CHAIRMAN'S REPORT, WHICH TODAY FOCUSES ENTIRELY ON THE MEMORY OF OUR WONDERFUL VICE CHAIR DUANE ROTH. WE HAVE WITH US DUANE'S WIFE RENEE AND BROTHER TED IN THE AUDIENCE. I WILL MAKE A FEW INTRODUCTORY COMMENTS. WE'LL THEN PROCEED TO A VIDEO THAT WE HAD IN DUANE'S HONOR AND FOLLOW THAT WITH COMMENTS BY MEMBERS OF THE BOARD AND THOSE IN THE AUDIENCE WHO WOULD LIKE TO SPEAK.

(THE TRIBUTE TO DUANE ROTH WAS THEN HEARD, NOT REPORTED NOR HEREIN TRANSCRIBED. DURING THE TRIBUTE A MOTION WAS MADE, SECONDED, AND APPROVED UNANIMOUSLY TO NAME THE DISEASE TEAM III AWARDS THE "DUANE ROTH DISEASE TEAM III ROUND OF FUNDING.")

CHAIRMAN THOMAS: WE ARE NOW GOING TO MOVE ON TO ITEM 7 ON OUR AGENDA. BACK TO THE WORK, THE WONDERFUL WORK, THAT CIRM DOES AND THE OUTSTANDING SCIENTISTS THAT IT FUNDS. THIS IS CONSIDERATION OF APPLICATIONS FOR RFA 12-07, CIRM EARLY TRANSLATIONAL IV RESEARCH AWARDS. DR. COLLINS PRESENTING.

DR. COLLINS: GOOD MORNING, MR. CHAIRMAN, MEMBERS OF THE BOARD, AND AUDIENCE. TODAY I'D LIKE TO PRESENT TO YOU THE RESULTS OF THE GRANTS WORKING GROUP REVIEW OF THE FOURTH CALL OF OUR EARLY

TRANSLATIONAL RFA. AND BEFORE I BEGIN, I'D LIKE TO JUST REFRESH YOU BRIEFLY REGARDING THE KEY FEATURES OF THE RFA, WHICH WAS APPROVED AT OUR CONCEPT DISCUSSION LAST AUGUST. SO IT'S BEEN A LITTLE WHILE SINCE WE SPOKE ABOUT IT.

AS YOU RECALL, THE GOAL OF THIS RFA IS REALLY TO BRIDGE THAT SPACE BETWEEN BASIC RESEARCH DISCOVERIES AND PRECLINICAL DEVELOPMENT. AND THIS IS A REALLY IMPORTANT ACTUALLY CRITICAL PERIOD IN THE PROJECT'S LIFETIME BECAUSE IT'S HERE WHEN WE FIND WHETHER A PROPOSED THERAPEUTIC APPROACH REALLY COULD STAND UP TO THE RIGORS REQUIRED TO MOVE ON TOWARDS THE CLINIC.

AS YOU MAY RECALL, WE OFFER TWO TYPES OF AWARDS, THE DEVELOPMENT CANDIDATE AWARDS AND THE DEVELOPMENT CANDIDATE FEASIBILITY AWARDS. AND I'LL REFER TO THESE AS DC AND DCF AWARDS. AND THESE TWO AWARDS ACTUALLY HAVE FAIRLY DIFFERENT END GOALS.

I THINK OF THE DEVELOPMENT CANDIDATE FEASIBILITY AWARDS AS THE HYPOTHESIS-DRIVEN PROJECTS WITH AN ULTIMATE EYE TOWARDS TRANSLATION. SO REALLY WHAT THESE AWARDS ARE DOING IS TESTING A THERAPEUTIC HYPOTHESIS WHILE, IN CONTRAST, THE DEVELOPMENT CANDIDATE AWARDS ARE REALLY GEARED TO ACHIEVING A DEVELOPMENT CANDIDATE. SO I THINK OF THESE AS

TRANSLATIONAL AWARDS WITH A DEFINED SET OF GOALS AND OUTCOMES. AND I'LL JUST GO OVER THOSE IN THIS SLIDE HERE TO LET YOU KNOW THE KIND OF EXPECTATIONS THAT WE HAVE FOR THESE DEVELOPMENT CANDIDATE AWARDS.

SO I THINK OF IT THIS WAY. WE'RE LOOKING FOR A THERAPEUTIC CANDIDATE THAT YOU MIGHT BE ABLE TO USE IN HUMANS. WE WANT IT TO BE WELL CHARACTERIZED. SO YOU NEED TO KNOW WHAT EACH LOT OF THAT CANDIDATE SHOULD LOOK LIKE. WE'RE LOOKING TO SEE WHETHER IT MIGHT IMPACT DISEASE SOMEDAY, SO WE'RE LOOKING FOR REPRODUCIBLE EVIDENCE OF DISEASE MODIFYING ACTIVITY. WE WANT TO KNOW HOW IT MIGHT WORK. WE'D LIKE SOME PRELIMINARY ASSESSMENT OF THE SAFETY AND HOW YOU MIGHT DELIVER THIS THERAPY.

IN ADDITION, WE WANT TO BE ABLE TO HAVE SOME EVIDENCE THAT ONE CAN MAKE THIS CANDIDATE CONSISTENTLY AND IN A METHOD OR USING REAGENTS THAT WOULD BE SUITABLE FOR HUMAN USE. AND WE WANT TO BE ABLE TO HAVE SOME SCALE-UP OF THIS PROCESS. SO DOES IT WORK? DO YOU KNOW WHAT IT LOOKS LIKE? HOW DOES IT WORK? AND CAN YOU MAKE IT?

I'D LIKE TO HIGHLIGHT THE TYPES OF THINGS THAT REVIEWERS EVALUATED DURING THE REVIEW. REALLY FOR FEASIBILITY THEY WERE LOOKING FOR COMPELLING PRELIMINARY DATA TO SUPPORT THE PROPOSED APPROACH.

WE'RE ALSO LOOKING FOR A COMPLETE PLAN TO ADDRESS THE GOALS OF THE APPLICATION. SO, FOR EXAMPLE, FOR A DEVELOPMENT CANDIDATE AWARD, WERE ALL THOSE ACTIVITIES ADDRESSED, ALL THE REQUIREMENTS IN THE PREVIOUS SLIDE, WERE THOSE ADDRESSED? FOR A DEVELOPMENT CANDIDATE FEASIBILITY AWARD, WE'D BE LOOKING FOR SOME EVIDENCE OF PROOF OF CONCEPT FOR THE HYPOTHESIS. FOR EXAMPLE, THIS COULD BE IN AN IN VITRO MODEL OF DISEASE OR AN IN VIVO MODEL.

FOR THE OBJECTIVE, RATIONALE, AND IMPACT, THEY WERE LOOKING TO SEE IF THIS MIGHT SOMEDAY PROGRESS TO BE A THERAPY THAT MIGHT HELP PATIENTS.

FOR THE TEAM, OBVIOUSLY WE'RE LOOKING FOR AN APPROPRIATE TEAM WITH THE EXPERTISE THAT'S REQUIRED TO PURSUE THE OBJECTIVE OF THAT PROPOSAL. AND I'D LIKE TO HIGHLIGHT THAT FOR THE DEVELOPMENT CANDIDATE AWARDS, PRODUCT DEVELOPMENT EXPERIENCE WAS HELPFUL, AND REVIEWERS WERE LOOKING FOR THAT AS WELL.

FINALLY, WE WERE LOOKING FOR APPROPRIATE STEM CELL PROJECTS AND SUPPORTIVE ENVIRONMENT FOR TRANSLATIONAL RESEARCH. WE ASKED REVIEWERS TO PRIORITIZE THESE TYPES OF PROGRAMS, CELL THERAPY, POTENTIALLY TRANSFORMATIVE THERAPEUTIC APPROACHES, AND PROJECTS THAT WERE ADDRESSING DISEASES PREVALENT

IN THE PEDIATRIC PATIENTS.

THESE ARE ELIGIBILITY CRITERIA. THEY'RE FAIRLY STANDARD. THESE HAVEN'T CHANGED IN THE PAST SEVERAL ROUNDS. I'D JUST LIKE TO HIGHLIGHT THAT A CO-PI WAS AN OPTION FOR DEVELOPMENT CANDIDATE AWARDS AND THAT THE RFA WAS OPEN TO OUR COLLABORATIVE FUNDING PARTNER PROGRAM.

YOU APPROVED A TOTAL BUDGET OF UP TO $70 MILLION FOR THIS RFA AT APPROXIMATELY TEN AWARDS OF EACH TYPE, THE DEVELOPMENT CANDIDATE AND THE DEVELOPMENT CANDIDATE FEASIBILITY, AND THESE HAD BUDGETS OF UP TO $3.5 MILLION IN DIRECT PROJECT COSTS FOR THE CANDIDATE AND 1.2 MILLION FOR THE FEASIBILITY AWARDS. AND I'D ALSO LIKE TO NOTE THAT A LOAN OPTION WAS AVAILABLE FOR FOR-PROFITS IN THE DEVELOPMENT CANDIDATE CATEGORY.

FINALLY, I'D LIKE TO HIGHLIGHT OUR NEW SCORING SYSTEM. SO THE MAIN DIFFERENCE IN THIS SCORING SYSTEM IS THAT REVIEWERS WERE INSTRUCTED THAT THEIR SCORES WOULD ACTUALLY DETERMINE THE FUNDING RECOMMENDATION TO YOU. SO THERE WERE THREE TIERS OF SCORING THAT ARE PRESENTED HERE. AND SO THIS IS A LITTLE BIT DIFFERENT FROM OUR PREVIOUS SCORING SYSTEM, SO I'D JUST LIKE TO HIGHLIGHT THAT. AND REVIEWERS WERE AWARE OF THIS NEW SYSTEM. AND

THE PANEL DID RECOMMEND SIX DEVELOPMENT CANDIDATE FEASIBILITY AWARDS AND FIVE DEVELOPMENT CANDIDATE AWARDS.

UNLESS THERE ARE ANY QUESTIONS ABOUT THE RFA, I'D LIKE TO TURN THE NEXT STEP OVER TO DR. SAMBRANO.

CHAIRMAN THOMAS: ACTUALLY THANK YOU, DR. COLLINS. BEFORE WE GET TO THAT, WANTED TO NOTE THAT THIS IS REALLY THE FIRST MAJOR ROUND OF AWARDS WHICH UTILIZES A NEW PROTOCOL THAT WAS PUT IN PLACE EARLIER THIS YEAR WITH RESPECT TO APPEALS, RECONSIDERATION, ETC. AND I WOULD LIKE MR. HARRISON TO ADDRESS THE BOARD AND THE AUDIENCE JUST SO THAT EVERYBODY UNDERSTANDS THAT PROTOCOL AS IT APPLIES TO THIS SERIES OF AWARDS.

MR. HARRISON: GOOD MORNING. AS J.T. MENTIONED, THE BOARD SUBSTANTIALLY REVISED SOME OF ITS PROCEDURES APPLICABLE TO THE REVIEW OF APPLICATIONS IN MARCH. SINCE THIS IS THE FIRST MAJOR ROUND OF APPLICATIONS COMING TO THE BOARD FOR ITS CONSIDERATION, WE THOUGHT IT WOULD BE HELPFUL TO REMIND THE BOARD OF SOME OF THE DETAILS RELATING TO THOSE POLICY CHANGES, THE FIRST OF WHICH IS THAT THE BOARD WILL NO LONGER BE MAKING DECISIONS ON APPLICATIONS. INSTEAD, UNDER THE BYLAWS WE HAVE

ESTABLISHED WHAT IS CALLED THE APPLICATIONS REVIEW SUBCOMMITTEE, WHICH IS COMPOSED OF THE BOARDS'S TEN PATIENT ADVOCATE MEMBERS, FOUR LIFE SCIENCE COMMERCIAL MEMBERS, AND THE CHAIR AND STATUTORY VICE CHAIR. BECAUSE OF CURRENT VACANCIES, THERE ARE CURRENTLY 14 MEMBERS OF THE APPLICATION REVIEW SUBCOMMITTEE.

FOR THE 13 OF YOU WHO ARE APPOINTED FROM INSTITUTIONS THAT ARE ELIGIBLE FOR CIRM FUNDING, YOU ARE, UNDER THE BYLAWS, CONSIDERED EX OFFICIO MEMBERS OF THE APPLICATION REVIEW SUBCOMMITTEE, WHICH MEANS THAT YOU WILL HAVE THE OPPORTUNITY TO PARTICIPATE IN THE DISCUSSION OF APPLICATIONS AND TO OFFER YOUR COMMENTS PROVIDED THAT YOU DO NOT OTHERWISE HAVE A CONFLICT WITH RESPECT TO THE APPLICATION, BUT YOU WILL NOT BE CALLED UPON TO VOTE ON ANY OF THE APPLICATIONS.

AS IS OUR NORMAL PRACTICE, WE'VE PROVIDED EACH OF YOU WITH A LIST OF APPLICATIONS BY APPLICATION NUMBER OF THOSE APPLICATIONS IN WHICH YOU HAVE A CONFLICT OF INTEREST. AND WE WOULD REMIND YOU TO CONSULT THAT LIST BEFORE RAISING YOUR HAND TO COMMENT ON A SPECIFIC APPLICATION.

AS LILA SAID, THERE WERE ALSO CHANGES TO THE GRANTS WORKING GROUP PROCESS AS WELL, ONE OF

WHICH IS THAT WE HAVE NOW DEFINED THE FUNDING TIERS IN ADVANCE OF THE GWG MEETING. SO THE SCIENTISTS WHO WERE ASSIGNING SCORES TO THE APPLICATIONS KNOW THAT IF THEY'RE SCORING AN APPLICATION BETWEEN 75 AND A HUNDRED, IT MEANS THAT THEY BELIEVE THAT APPLICATION SHOULD BE FUNDED. FOR WHAT WE CALL TIER II, SCORES OF 65 TO 74, WE'VE EXPLAINED TO THE MEMBERS OF THE GRANTS WORKING GROUP THAT TIER II REPRESENTS APPLICATIONS THAT ARE JUDGED TO BE OF MODERATE SCIENTIFIC QUALITY OR APPLICATIONS WHERE CONSENSUS ON SCIENTIFIC MERIT CANNOT BE REACHED, AND THESE APPLICATIONS MAY BE SUITABLE FOR THE BOARD'S PROGRAMMATIC CONSIDERATION.

THAT'S, OF COURSE, THE OTHER SIGNIFICANT FEATURE OF THE POLICY CHANGE. PROGRAMMATIC REVIEW, WHICH WAS FORMERLY CONDUCTED AT THE GRANTS WORKING GROUP, HAS NOW BEEN TRANSFERRED TO THE APPLICATION REVIEW SUBCOMMITTEE.

THE OTHER NOTABLE CHANGE IN THIS PROCESS IS THAT THE BOARD, BOTH IN ITS BYLAWS AND IN THE GRANTS WORKING GROUP BYLAWS, EMPOWERED STAFF TO MAKE ANY ADDITIONAL RECOMMENDATIONS THEY HAVE WITH RESPECT TO APPLICATIONS BEYOND THOSE OF THE GRANTS WORKING GROUP.

FOLLOWING DR. SAMBRANO'S PRESENTATION OF

THE SCORES, DR. OLSON WILL PRESENT THOSE STAFF RECOMMENDATIONS TO THE BOARD. MR. SHEEHY WILL THEN MODERATE THE APPLICATION REVIEW SUBCOMMITTEE'S PROGRAMMATIC CONSIDERATION OF APPLICATIONS.

JUST AS A REMINDER, PROGRAMMATIC CONSIDERATION WAS INTENDED TO ENCOMPASS NONSCIENTIFIC FACTORS SUCH AS PORTFOLIO BALANCE, RELEVANCE TO UNMET HEALTH NEEDS, THE URGENCY OF THE TIMELINE, ALIGNMENT WITH THE FOCUS OF PROP 71, AND ALIGNMENT WITH THE GOALS AND PRIORITIES OF THE REQUEST FOR APPLICATIONS. PROGRAMMATIC CONSIDERATION WILL OBVIOUSLY ALSO TAKE INTO CONSIDERATION THE STAFF RECOMMENDATIONS AND ANY PUBLIC COMMENT.

MEMBERS OF THE SUBCOMMITTEE WHO ARE ELIGIBLE TO VOTE MAY MAKE MOTIONS TO MOVE AN APPLICATION FROM ONE TIER TO ANOTHER. AND WE WILL INVITE MEMBERS OF THE PUBLIC WHO WISH TO MAKE A COMMENT WITH RESPECT TO AN APPLICATION THAT IS THE SUBJECT OF A MOTION TO OFFER THAT COMMENT AT THAT TIME. ONCE ALL THE MOTIONS HAVE BEEN EXHAUSTED, MR. SHEEHY WILL ASK FOR A MOTION TO APPROVE FUNDING FOR THOSE APPLICATIONS IN TIER I AND TO CLOSE FUNDING FOR THOSE APPLICATIONS THAT REMAIN. AT THAT POINT IN TIME, WE'LL INVITE PUBLIC COMMENT FROM MEMBERS OF

THE PUBLIC WITH RESPECT TO ANY APPLICATION THAT HAS NOT BEEN THE SUBJECT OF AN INDIVIDUAL MOTION.

JUST BRIEFLY, TO REMIND THE BOARD, WE HAVE REPEALED THE EXTRAORDINARY PETITION POLICY WHICH FORMERLY PERMITTED APPLICANTS TO CORRESPOND DIRECTLY WITH THE BOARD ON SCIENTIFIC ISSUES RELATING TO THEIR APPLICATION. IN ITS PLACE THE BOARD ADOPTED AN APPEAL AND REQUEST FOR RECONSIDERATION POLICY PURSUANT TO WHICH APPLICANTS HAVE THE OPTION TO DIRECT AN APPEAL OR REQUEST FOR RECONSIDERATION TO CIRM STAFF BASED EITHER ON A MATERIAL DISPUTE OF FACT OR A REQUEST FOR RECONSIDERATION BASED ON MATERIAL NEW INFORMATION.

UNDER THIS POLICY CIRM STAFF REVIEWS THE APPEAL OR REQUEST FOR CONSIDERATION TO DETERMINE WHETHER OR NOT THE APPLICANT HAS SET FORTH CLEAR GROUNDS ESTABLISHING THE OCCURRENCE OF EITHER A DISPUTE OF FACT OR MATERIAL NEW INFORMATION. AND IF THE APPLICANT HAS NOT SET FORTH SUCH GROUNDS FOR AN APPEAL, OR IF THE PRESIDENT DETERMINES THAT IT WOULD NOT HAVE AFFECTED THE OUTCOME, THE APPEAL IS DENIED AND THE GRANTS WORKING GROUP'S RECOMMENDATION IS PRESENTED TO THE BOARD AS IT WAS AT THE GRANTS WORKING GROUP.

BY CONTRAST, IF THE STAFF DETERMINES THAT

THE APPLICANT HAS MET THE SHOWING AND THE PRESIDENT DETERMINES THAT ADDITIONAL SCIENTIFIC REVIEW IS WARRANTED, THEN THE BOARD'S CONSIDERATION OF THAT APPLICATION WILL BE DEFERRED UNTIL THAT REVIEW HAS OCCURRED.

IN THIS PARTICULAR CASE, AS ALWAYS, CIRM STAFF HAS ADVISED APPLICANTS OF THEIR OPTION TO FILE AN APPEAL OR A REQUEST FOR RECONSIDERATION. AND FIVE APPLICANTS FOR EARLY TRANSLATION IV APPLICATIONS SUBMITTED SUCH APPEALS. THE STAFF'S ACTION ON THOSE APPEALS IS IN YOUR MATERIALS.

MEMBERS OF THE PUBLIC, OF COURSE, REMAIN FREE TO OFFER PUBLIC COMMENT, AND THAT INCLUDES APPLICANTS, SOME OF WHOM YOU MAY HEAR TODAY. SO IF YOU HAVE ANY QUESTIONS, CHAIR, I'D BE HAPPY TO ANSWER THEM.

CHAIRMAN THOMAS: QUESTIONS FROM MEMBERS OF THE BOARD? I DO WANT TO HIGHLIGHT ONE THING JAMES SAID BECAUSE IT'S VERY IMPORTANT. THE NEW APPELLATE PROCEDURE WAS PUT IN PLACE SPECIFICALLY SO THAT APPEALS ON PARTICULAR PROJECTS WOULD NOT BE HEARD AS A MATTER OF FIRST INSTANCE AT THE BOARD. WE WERE STRIVING TO GET AWAY FROM THAT BECAUSE THAT PUT THE BOARD IN THE POSITION OF HAVING TO MAKE DECISIONS ON THE SPOT WHICH THEY WERE NOT REALLY

PREPARED TO DO BASED ON HAVING ADEQUATE BACKGROUND, REVIEW, ETC., AND THAT'S WHY APPEALS WERE DIRECTED PROPERLY TO STAFF WHO WOULD DO A FULL INVESTIGATION AND REVIEW AND COME BACK TO US WITH RECOMMENDATIONS.

SO TO THE EXTENT THAT GOING FORWARD, NOT JUST TODAY, APPEALS ARE NOT MADE THROUGH THAT PROCESS, BUT ARE MADE AS A MATTER OF FIRST INSTANCE HERE AT THE BOARD MEETING, THAT PUTS AN EXCEPTIONALLY HIGH BURDEN ON WHOEVER IS MAKING THAT APPEAL AND IS NOT THE PREFERRED WAY TO GO. SO I WANT TO BE VERY CLEAR ABOUT THAT. WE HAVE THIS, I THINK, VERY GOOD PROTOCOL IN PLACE, AND WE NEED TO BE AWARE OF THAT. YOU WILL SEE HOW THAT HAS WORKED WHEN WE GET A LITTLE BIT LATER IN A FEW MINUTES TO SOME OF THE PROJECTS THAT HAVE BEEN REVIEWED BY STAFF.

DR. LUBIN: SO I'D JUST LIKE TO MENTION THIS IS SOMETHING THAT DUANE WAS VERY INVOLVED IN. I TRIED TO HELP WITH THAT A LITTLE, AND IT EVOLVED TO THE PROCESS THAT WE HAVE NOW. AND IT'S REMARKABLE THAT ON TODAY WITH ALL THE HONORING OF DUANE THIS IS ONE OF THE MAJOR STEPS THAT HE MADE RECENTLY. AND I JUST WANTED TO LET THE BOARD KNOW ABOUT THAT.

CHAIRMAN THOMAS: THANK YOU, DR. LUBIN.

AND THAT SORT OF COMMENT HAS BEEN MADE REPEATEDLY WITH RESPECT TO ALL SORTS OF STUFF, AS WAS SAID. DUANE SAID THIS. DUANE IS STILL HERE AND WILL ALWAYS REMAIN HERE AS WE MOVE FORWARD.

OKAY. SO HAVING HEARD THE PROTOCOL, NOW LET'S TURN TO DR. SAMBRANO.

DR. SAMBRANO: THANK YOU VERY MUCH. ALL I REALLY NEED TO DO HERE IS GUIDE YOU THROUGH WHAT I'M PRESENTING ON THE SCREEN, WHICH ARE THE RECOMMENDATIONS FROM THE GRANTS WORKING GROUP SHOWN IN THEIR RESPECTIVE TIERS FOR THE TWO AWARDS TYPES.

IT'S GOING TO BE PERHAPS DIFFICULT TO SEE ON THE SCREEN, BUT ONE OF THE THINGS I WANT TO EXPLAIN IS THAT THE GRANTS WORKING GROUP REVIEWED THE DCF, THOSE THAT WERE DEVELOPMENT CANDIDATE FEASIBILITY AWARDS, SEPARATE FROM THE DEVELOPMENT CANDIDATE AWARDS. SO THERE ARE TWO SETS OF TIERS.

SO MY RECOMMENDATION TO YOU IS THAT, SINCE THE GRANTS WORKING GROUP CONSIDERED THE DCF APPLICATIONS FIRST, THAT PERHAPS THE BOARD CONSIDER THEM IN THAT ORDER AS WELL AND THEN DO THE DC APPLICATIONS.

FOR THE DCF, YOU WILL NOTICE THAT THERE ARE THREE APPLICATIONS THAT FELL IN TIER II. FOR THOSE WE HAVE STAFF RECOMMENDATIONS THAT DR. PAT

OLSON WILL DESCRIBE TO YOU. FOR THE DC AWARDS, THE GRANTS WORKING GROUP DID NOT PLACE ANY APPLICATIONS IN TIER II. SO THERE IS A DISTINCTION BETWEEN THOSE THAT ACTUALLY FELL IN TIER I THAT SCORED 75 OR ABOVE AND THEN THOSE THAT WERE BELOW 64. THERE JUST HAPPENED TO BE NONE THAT FELL IN TIER II.

SO IT'S UP TO YOU HOW YOU WISH TO CONSIDER THESE APPLICATIONS, BUT MY SUGGESTION IS THAT YOU BEGIN WITH THE DCF AS CURRENTLY SHOWN ON THE SCREEN. AND DR. OLSON WILL PRESENT THE RECOMMENDATIONS FROM STAFF ON THOSE THAT ARE IN TIER II.

DR. OLSON: MEMBERS OF THE BOARD AND THE PUBLIC, THANK YOU FOR THIS OPPORTUNITY. STAFF, WORKING WITH THE PRESIDENT, HAS ACTUALLY SPENT CONSIDERABLE TIME AND BRINGS THE FOLLOWING FORTH FOR YOUR CONSIDERATION. I'D LIKE TO JUST GO THROUGH THESE IN ORDER.

SO APPLICATION NUMBER TR4-0666, THIS IS AN APPLICATION. IT IS A DCF FEASIBILITY AWARD. IT IS A TIER II AWARD. IT RECEIVED AN AVERAGE SCORE OF 70. THE TITLE OF THIS AWARD IS "HUMAN PLURIPOTENT STEM CELL-DERIVED PHOTORECEPTORS FOR RETINAL DEGENERATIVE DISORDERS." SO I'LL TELL YOU THAT THE DISEASE TARGET IS THOSE RETINAL DEGENERATIVE DISORDERS SUCH AS RETINITIS PIGMENTOSA OR CERTAIN

CONGENITAL RETINAL DISEASE SUCH AS X-LINK CONE OR CONE-ROD DISEASE.

THE APPROACH HERE IS AN ALLOGENEIC APPROACH THAT IS AN OFF-THE-SHELF CELL THERAPY, AND IT'S EITHER HESC DERIVED, EMBRYONIC STEM CELL-DERIVED, OR HIPSC DERIVED, INDUCED PLURIPOTENT CELL-DERIVED PHOTORECEPTORS. SO IT'S THE ACTUAL TARGET CELL. THE REQUESTED FUNDING WAS $1.96 MILLION.

THE POINTS THAT WE WOULD LIKE TO RAISE FOR YOUR CONSIDERATION IS THAT CIRM IS CURRENTLY FUNDING TWO SIMILAR -- CURRENTLY IS OR WILL BE OR IF YOU ACCEPT THE GRANTS WORKING GROUP RECOMMENDATIONS ON THIS ROUND, WE'RE CURRENTLY FUNDING ONE APPROACH TO THIS, AND THERE IS A HIGHLY RECOMMENDED OR A RECOMMENDED SECOND APPROACH TO THIS. SO ADDITIONAL INVESTMENT OF AN EARLIER STAGE PROJECT IS A LITTLE BIT HARDER TO JUSTIFY.

THE ONE APPROACH IS ACTUALLY A DISEASE TEAM APPROACH, WHICH IS AN ALLOGENEIC APPROACH USING RETINAL PROGENITOR CELLS AS OPPOSED TO THE FULLY DIFFERENTIATED CELLS THAT ARE DERIVED FROM TISSUE STEM CELLS. THIS ONE HAS A GOAL OF IND FILING AND COMPLETION OF A PHASE I-II TRIAL.

THE ONE THAT IS UP FOR YOUR CONSIDERATION

TODAY, WHICH IS RECOMMENDED BY THE GRANTS WORKING GROUP, IS AN ALLOGENEIC APPROACH USING ESSENTIALLY A 3D STRUCTURE. IT'S HESC-DERIVED SHEETS OF PROGENITOR CELLS, AND IT ALSO INCLUDES RETINAL EPITHELIAL CELLS. AND THIS PROJECT HAS A GOAL OF IDENTIFYING A DEVELOPMENT CANDIDATE AWARD.

SO BASED ON THE FACT THAT WE MAY HAVE TWO PROJECTS, WE CERTAINLY HAVE ONE PROJECT, TARGETING VERY SIMILAR APPROACHES, OUR RECOMMENDATION TO YOU IS TO NOT FUND THIS AWARD.

THE SECOND AWARD THAT WE'D LIKE TO YOU TO CONSIDER IS THE TR4-06823, ALSO A FEASIBILITY AWARD ALSO IN TIER II WITH AN AVERAGE SCORE OF 69. THIS AWARD IS ENTITLED "BETA GLOBIN GENE CORRECTION OF SICKLE CELL DISEASE IN HEMATOPOIETIC STEM CELLS." THE TARGET IS SICKLE CELL DISEASE. THE APPROACH IS AUTOLOGOUS HEMATOPOIETIC STEM CELLS -- SO AUTOLOGOUS MEANS THE PATIENTS THEMSELVES, THEIR CELLS -- GENETICALLY MODIFIED EX VIVO TO ACTUALLY CORRECT THE MUTATION IN THE BETA GLOBIN GENE. THE REQUESTED FUNDING FOR THIS AWARD IS 1.815 MILLION.

AND THE POINTS -- THIS ONE WE'RE ACTUALLY RECOMMENDING FOR YOUR CONSIDERATION WITH A CONDITION. AND THE POINTS THAT I'D LIKE TO RAISE FOR CONSIDERATION IS WE ARE FUNDING ANOTHER PROJECT

IN SICKLE CELL DISEASE THAT INVOLVES AN ADDITION THROUGH LENTIVIRAL MEDIATED GENE ADDITION OF A FETAL BETA GLOBIN GENE. SO IT HAS A VIRUS INTEGRATION AND IT ADDS A GENE. BUT IT DOES -- WHAT THIS ONE DOES IS IT LEVERAGES THE TEAM AND THE KNOW-HOW THAT IS GAINED IN THAT RELATED PROJECT. IT'S LIKELY, BECAUSE OF THE TECHNOLOGY INVOLVED, TO ENABLE A RAPID PATH TO THE CLINIC FOR A RELATIVELY LOW INVESTMENT OF $1.8 MILLION.

AS I SAID, THE OTHER PROJECT IS DIFFERENT. THE APPROACH HERE IS ACTUALLY A GENE CORRECTION AS OPPOSED TO ANOTHER GENE ADDITION. THE REVIEWERS HAD -- I THINK EVERYBODY DOESN'T KNOW FOR SURE WHAT IT'S GOING TO TAKE TO CORRECT THE SYMPTOMS, BUT BASED ON ALLOGENEIC BONE MARROW TRANSPLANTATION, PEOPLE BELIEVE BETWEEN 10 AND 20 PERCENT. I THINK WE CAN DEAL WITH THAT IN THE MILESTONES.

I WOULD LIKE TO NOTE, AND THIS IS THE BASIS FOR OUR CONDITION, WHILE THE PROJECT IS LEVERAGED BY AN IN-KIND CONTRIBUTION OF ESSENTIAL SERVICES AND TECHNOLOGY AND EXPERTISE, IT'S PART OF THE PROPOSAL, THE PARTNER IS PART OF PROPOSAL, BUT THEY DON'T SHOW UP IN TERMS OF ANY KIND OF FINANCIAL CONTRIBUTION OR ANYTHING LIKE THAT. SO THESE ARE KEY -- IT'S A KEY COLLABORATOR. THIS PROJECT CANNOT

BE DONE WITHOUT THIS COLLABORATION.

AND SO WE WOULD LIKE TO RECOMMEND THAT YOU FUND WITH A CONDITION WHICH INCLUDES EXECUTION OF A FORMALIZED AGREEMENT WITH THE KEY PROJECT COLLABORATOR AND THAT THAT AGREEMENT IS TO THE SATISFACTION OF CIRM STAFF.

THIS ESSENTIALLY ACKNOWLEDGES THE FACT THAT IT'S A CO-FUNDED PROJECT ALMOST AND THAT THE PROJECT CANNOT BE DONE WITHOUT THE KEY CORPORATE COLLABORATOR.

THE SECOND IS APPLICATION -- OR THE FINAL ONE THAT WE WANT TO BRING TO YOUR ATTENTION IS APPLICATION TR4-06831, AGAIN A FEASIBILITY AWARD, TIER II, WITH AN AVERAGE SCORE OF 66.

THE TITLE OF THIS AWARD IS "GENE THERAPY CORRECTED AUTOLOGOUS HEPATOCYTE-LIKE CELLS FROM INDUCED PLURIPOTENT STEM CELLS FOR TREATMENT OF PEDIATRIC SINGLE ENZYME DISORDERS." SO IT'S TARGETING UREA CYCLE DISORDER. AND THE APPROACH IS AUTOLOGOUS, SO, AGAIN, PATIENT'S OWN CELLS, CONVERTED TO IPSC THAT ARE GENETICALLY MODIFIED EX VIVO TO CORRECT A MUTANT ENZYMES GENE AND THEN DIFFERENTIATED TO HEPATOCYTE-LIKE CELLS FOR TRANSPLANTATION.

IT'S $1.8 MILLION. AND THE POINTS THAT --

THIS ONE WE ACTUALLY ARE ALSO RECOMMENDING, AND THE POINTS FOR CONSIDERATION HERE IS THAT IT IS A DISEASE. THIS PARTICULAR DISEASE IS ONE WHERE THE PERCENTAGE OF ENGRAFTED CORRECTED CELLS REQUIRED TO MAKE A DIFFERENCE FOR DISEASE MODIFICATION IS LOW. AND WHEN YOU THINK ABOUT IT, YOU HAVE TO REALLY EFFICIENTLY TRANSDUCE CELLS TO GET THE CORRECTION AND YOU HAVE TO ENGRAFT. SO THIS LOWER HURDLE IS ACTUALLY AN IMPORTANT TECHNICAL CONSIDERATION.

ALSO, ALTHOUGH CIRM HAS THREE OTHER PROJECTS IN ITS TRANSLATIONAL PORTFOLIO, THEY TARGET OTHER LIVER DISEASES AND SEEK TO GENERATE HEPATOCYTE-LIKE CELLS FROM DIFFERENT SOURCES AND BY DIFFERENT APPROACHES. SO THE SUCCESSFUL GENERATION OF HEPATOCYTE-LIKE CELLS ACTUALLY WOULD BE A VERY GOOD THING FOR THE FIELD. THERE'S A LOT OF THINGS YOU COULD DO. SO WE THINK THAT HAVING SEVERAL INVESTIGATORS PURSUING SEVERAL DIFFERENT APPROACHES HERE IS REALLY GOOD. AND IT'S ALSO TRUE THAT A VERY RECENT PUBLICATION HAS COME OUT THAT SUGGESTS AN INCREASED LIKELIHOOD OF GETTING THESE HEPATOCYTE-LIKE CELLS, THAT THERE ARE METHODS NOW THAT PEOPLE ARE FINDING INVOLVING LIVER BUD CELLS THAT WOULD REALLY HELP.

SO THOSE ARE THE STAFF POINTS THAT WE'D

LIKE TO RAISE FOR YOUR CONSIDERATION. AND IF ANYONE HAS ANY QUESTIONS, I'LL BE HAPPY TO ANSWER THEM. THANK YOU.

CHAIRMAN THOMAS: THANK YOU, DR. OLSON. LET'S TURN IT OVER NOW TO PROGRAMMATIC REVIEW TO MR. SHEEHY. MR. HARRISON.

MR. HARRISON: I WAS JUST GOING TO SUGGEST WE TAKE A FIVE-MINUTE BREAK.

CHAIRMAN THOMAS: FIVE-MINUTE BREAK BEFORE WE GET TO PROGRAMMATIC.

(A RECESS WAS TAKEN.)

CHAIRMAN THOMAS: COULD EVERYBODY PLEASE TAKE THEIR SEATS?

(THE APPLICATION REVIEW SUBCOMMITTEE WAS THEN CONVENED AND HEARD AS FOLLOWS:)

MR. SHEEHY: IS EVERYONE BACK AND READY TO START? YES. SO TWO THINGS. ONE, I KNOW WE'RE GOING TO BE SAYING THIS AD INFINITUM, BUT I THINK DUANE WOULD BE VERY PROUD OF HOW WE'VE IMPLEMENTED THESE CHANGES IN OUR PROCESS. HE SPOKE OFTEN ABOUT GETTING MORE STAFF INPUT ON GRANTS. AND I CERTAINLY APPRECIATE THE PRESENTATION THAT DR. OLSON DID AND THE WORK THAT STAFF HAS DONE IN LOOKING AT THESE GRANTS IN THE MIDDLE SECTION. AND I THINK THIS IS A VERY IMPORTANT FEATURE THAT WE'VE ADDED TO OUR

PROCESS, AND THAT WE'RE GIVING STAFF A CHANCE TO LOOK AT THESE GRANTS, APPLY A CONDITION IN ONE INSTANCE, RECOMMEND ANOTHER, DON'T RECOMMEND ANOTHER. BUT I JUST KNOW DUANE APPRECIATED SO MUCH THE HARD WORK AND THE EXPERTISE THAT WE HAVE HERE AT CIRM, AND REALLY PUTTING THAT BRAIN POWER TO WORK AT THIS POINT IN OUR PROCESS IS A GREAT INNOVATION. SO THANK YOU TO DUANE AND THANK YOU TO STAFF BECAUSE THIS IS SOMETHING THAT'S BEEN A LONG TIME IN THE WORKS.

THE OTHER THING THAT I THINK MIGHT BE HELPFUL IS THERE ARE TWO GRANTS THAT ARE RECOMMENDED FOR FUNDING THAT ARE OUTSIDE THE SCORING RANGES AS WE ESTABLISHED THEM. WE DID NOT HAVE PROGRAMMATIC REVIEW AT THE WORKING GROUP. SO I THINK PERHAPS -- I'VE ASKED DR. SAMBRANO TO GIVE US A LITTLE BIT OF A SENSE OF WHAT TOOK PLACE AND HOW THOSE GOT THERE BEFORE WE ACTUALLY GO INTO THE REVIEW.

DR. SAMBRANO: THANK YOU, MR. SHEEHY. YES, ABSOLUTELY. THERE ARE, IN FACT, TWO APPLICATIONS. ONE OF THEM IS 6648, WHICH HAS A SCORE OF 64, THAT YOU WILL FIND IN TIER I. YOU WILL ALSO FIND ONE APPLICATION, 6809, WITH A SCORE OF 73 THAT'S ALSO IN TIER I.

SO DURING THE FINAL PHASE OF REVIEW, WE

DID NOT HAVE A PROGRAMMATIC DISCUSSION, BUT WE DID HAVE AN ADJUSTMENT OF SCORE PERIOD WHERE THE WORKING GROUP WAS ABLE TO LOOK AT THE RANK ORDER OF APPLICATIONS AND MAKE ADJUSTMENTS AS NECESSARY.

SO MORE SPECIFICALLY, ON APPLICATION 6648, WHICH SCORED A 64, THAT APPLICATION WAS SCORED A 64 BECAUSE WE INSTRUCTED REVIEWERS THAT THEY SHOULD SCORE AS THE APPLICATION IS PRESENTED TO THE REVIEW GROUP BY THE APPLICANT. THEY FELT THAT THERE WAS A CRITICAL MILESTONE THAT THEY COULD ADD TO THAT PROPOSAL THAT, IN ESSENCE, WOULD HAVE CHANGED THE SCORE IN THEIR MIND AND MAKE IT A FUNDABLE PROPOSAL. SO ALTHOUGH THE SCORE IS A 64, IT ALSO INCLUDES VERY SPECIFICALLY A CONDITION THAT NOW PLACES IT IN TIER I.

THE OTHER PROPOSAL, WHICH IS 6809 WHICH SCORED 73, WAS LOOKED AT BECAUSE IT HAD A MEDIAN SCORE OF 75 WHICH THE GRANTS WORKING GROUP THOUGHT WAS ALREADY WELL WITHIN OR AT LEAST CLOSE ENOUGH TO THE UPPER TIER THAT IT WAS WORTH DISCUSSION. SOME OF THE REVIEWERS WHO WERE ASSIGNED TO THE APPLICATION EXPRESSED TO THE WORKING GROUP THAT THEIR SCORE -- THAT THE SCORE THEY INTENDED SHOULD HAVE BEEN HIGHER. AT THIS POINT THE SCORES HAD BEEN FIXED, SO THERE WAS NO WAY FOR THEM TO OTHERWISE DO

IT. THEY DISCUSSED THE PROPOSAL AND VOTED IN FAVOR OF PLACING IT WHERE THEY THOUGHT IT WAS MORE APPROPRIATE IN TIER I.

MR. SHEEHY: THANKS, DR. SAMBRANO. IN TERMS OF THE PROCESS, WHAT I WILL -- THE WAY IN WHICH WE DID IT AT THE WORKING GROUP AND I THINK THE WAY WE'LL PROCEED HERE IN PROGRAMMATIC REVIEW IS BASICALLY A GRANT IS NOT UNDER DISCUSSION UNLESS THERE'S A MOTION AND A SECOND TO MOVE IT FROM ONE CATEGORY TO ANOTHER. SO RATHER THAN JUST HAVING A GENERALIZED DISCUSSION ABOUT THIS GRANT OR THAT GRANT, THAT'S GENERALLY THE PROCESS WE GO THROUGH.

SO BEFORE WE START, THOUGH, I THINK JOAN SAMUELSON MAY HAVE HAD SOME COMMENTS THAT SHE WANTED TO MAKE.

MS. SAMUELSON: I HAVE DECIDED THAT, AS FAR AS THIS ROUND OF GRANTS IS CONCERNED, I'M GOING TO ABSTAIN ON ALL OF THE GRANTS. AND HERE'S MY REASON. WE'VE TALKED, ESPECIALLY AT THE LAST FEW MEETINGS, ABOUT THE ISSUE OF ASSESSMENT OF THE PORTFOLIO THAT WE HAVE IN LIGHT OF FUTURE FUNDING AND ALSO THE DECLINING FUNDING WE HAVE AVAILABLE. AND I'VE BECOME TOO UNCOMFORTABLE, I THINK, WITH THE AVAILABLE OPTIONS TO US AS A BOARD. AND IN A SITUATION WHICH, BECAUSE OF THE CHANGES IN OUR

PROCEDURES, AND FOR VERY GOOD REASONS, GIVING THE STAFF SOME IMPORTANT INPUT OVER THE MERIT OF THE INDIVIDUAL APPLICATIONS, OUR OPPORTUNITIES FOR EXERCISING OUR FIDUCIARY DUTY HAVE BEEN REDUCED, AND IT MAKES THEM ALL THE MORE IMPORTANT TO MY MIND. AND I AM NO LONGER COMFORTABLE WITHOUT CLEAR INFORMATION ABOUT THE PORTFOLIO WE HAVE ALREADY FUNDED AND THE EXTENT TO WHICH WE'VE ACCOMPLISHED ANY OF THE OBJECTIVES OF THE MISSION AND HOW THAT RELATES TO THESE GIVEN INDIVIDUAL APPLICATIONS AND THE AMOUNT OF MONEY WE HAVE LEFT.

I THINK ALL OF THAT HAS TO BE TAKEN INTO CONSIDERATION IN THE SAME CONTEXT, AND I DON'T FEEL WE'RE ABLE WITH THE VOTING OPTIONS WE HAVE NOW. SO THAT'S THE REASON FOR MY VOTE. OF COURSE, I HOPE I WON'T BE DOING THAT UNTIL THE END OF OUR EXISTENCE. I HOPE WE CAN GET THE INFORMATION THAT I FEEL WE NEED AND BE SUFFICIENTLY INFORMED THAT THEN WE CAN TURN TO INDIVIDUAL GRANTS AND BE CONFIDENT WE KNOW WE'RE GETTING THE BEST BANG FOR THE BUCK. THANK YOU.

MR. SHEEHY: THANK YOU, JOAN.

SO FIRST LOOKING AT THE DEVELOPMENT CANDIDATE FEASIBILITY BRACKETS, WE'LL TAKE THEM IN TWO DIFFERENT BRACKETS, THE DEVELOPMENT CANDIDATE

AND THE DEVELOPMENT CANDIDATE FEASIBILITY. SO THE FIRST AREA IN WHICH A MOTION WOULD BE APPROPRIATE IS IF THERE IS ANY DESIRE TO MOVE ANY OF THE APPLICATIONS THAT ARE IN THE GREEN, THAT IS THE FUNDABLE CATEGORY, OUT OF THAT CATEGORY.

AND IF THERE ARE NO MOTIONS TO DO SO, THEN WE MOVE INTO TIER II WHERE I NOTE WE HAVE STAFF RECOMMENDATIONS ON ALL THREE. ARE THERE ANY MOTIONS TO MOVE ANY OF THOSE GRANTS INTO TIER I? AND I'LL JUST ASSUME THAT FAILURE TO MOVE A GRANT INTO TIER I BY DEFAULT LEAVES THAT IN TIER III, WHICH IS UNFUNDABLE. SO WE DON'T HAVE TO ACTUALLY MOVE THINGS OUT OF FUNDABILITY.

DR. PRIETO: I'LL START THE DISCUSSION BY MAKING A MOTION TO MOVE GRANT 6823 INTO TIER I.

MR. SHEEHY: DO WE HAVE A SECOND?

CHAIRMAN THOMAS: I'LL SECOND THAT.

MR. SHEEHY: IS THERE ANY DISCUSSION ON THIS? WE DID JUST HAVE THE PRESENTATION BY DR. OLSON ON THE STAFF RECOMMENDATION TO APPROVE IT FOR FUNDING. IF THERE'S NO DISCUSSION, I'M HAPPY TO TAKE PUBLIC COMMENT.

MR. TORRES: ON THAT POINT, WAS THERE A SECOND RECOMMENDATION DR. OLSON MADE?

MR. SHEEHY: WE'RE JUST GOING GRANT BY

GRANT. I'M SORRY. I DIDN'T SEE YOU, DR. LUBIN.

DR. LUBIN: THIS IS JUST A CLARIFICATION. IF YOU'RE IN CONFLICT, YOU CAN'T COMMENT; IS THAT RIGHT?

MR. HARRISON: THAT'S CORRECT.

MR. SHEEHY: SO IF NO ONE ON THE BOARD WISHES TO COMMENT, I'M HAPPY TO ENTERTAIN PUBLIC COMMENT ON THIS GRANT, WHICH WOULD BE 6823. IS THERE ANYONE WHO WISHES TO MAKE A PUBLIC COMMENT HERE OR AT ANY OF THE SITES?

DR. PRIETO: I'D JUST LIKE TO ECHO WHAT DR. OLSON RECOMMENDED TO US AND REMIND THE BOARD THAT THIS IS A GRANT THAT STAFF IS RECOMMENDING FOR FUNDING CONTINUATION OF IMPORTANT WORK THAT WE HAVE FUNDED PREVIOUSLY, SOMETHING THAT WOULD HAVE VERY LARGE POTENTIAL IMPACT.

MR. SHEEHY: DR. TROUNSON.

DR. TROUNSON: SO, JEFF, JUST IN SUPPORT OF THE CONSIDERATIONS HERE. THIS IS A WAY OF CORRECTING FOR THE WRONG GENE; WHEREAS, THE OTHER STUDIES THAT WE'VE FUNDED WAS TO INSERT A FETAL GENE AND HOPE THAT THE FETAL GENE, THE BETA GLOBIN, THERE WOULD ACCOMMODATE THE PATIENT IN DUE COURSE AND ENABLE THEM TO EVADE THE SICKLE CELL DISEASE.

CORRECTING THE GENE, I THINK, IS A MUCH

MORE EFFECTIVE WAY BECAUSE YOU KNOW YOU'RE GOING TO GET THE ADULT GENE. I THINK THE KIND OF WORK THAT THEY'VE BEEN DOING GIVES US SOME CONFIDENCE THAT THEY CAN GET TO THE 10- TO 15-PERCENT CONVERSION OF CELLS, THE HEMATOPOIETIC STEM CELLS, WITH USING THE ZINC FINGER NUCLEASE TECHNOLOGY. SO WE FELT THAT THIS WAS ONE OF THOSE PROJECTS. IT'S QUITE LIKELY TO MOVE ALONG QUITE QUICKLY, PARTICULARLY IF WE ENSURE, AND THAT'S WHAT WE REALLY WANTED YOU TO TAKE NOTE, THAT THERE'S A CO-FUNDER THERE THAT IS A COMMERCIAL ENTITY. AND THEY SHOULD BE FORMALLY INCORPORATED AS A PARTNER. SO IT SUITS US AND IT SUITS THE AGENCY AND CALIFORNIA BETTER TO HAVE THEM RECOGNIZED AS A PARTNER.

MR. SHEEHY: THANK YOU, DR. TROUNSON. TO BE CLEAR, THE MOTION THAT'S BEEN MADE AND SECONDED IS TO ACCEPT THE STAFF RECOMMENDATION WHICH INCLUDES THE CONDITIONS THAT HAVE BEEN IMPOSED THERE.

DR. PETERSON: CAN I ASK A QUESTION? I LIKE THE PARADIGM. I LIKE THE GOAL OF THIS GRANT. AND THE ONLY THING THAT BOTHERED ME ABOUT IT, AS YOU READ THE, I BELIEVE IT'S, THE GWG ASSESSMENT, IT'S REPLETE WITH NEGATIVITY ABOUT THE POTENTIAL OF REACHING THAT 10- TO 15-PERCENT LEVEL THAT YOU CITE. AND SO THE ONLY QUESTION I HAD AS A REVIEWER WAS

WHAT PRELIMINARY EVIDENCE DID YOU HAVE THAT THAT, IN FACT, MIGHT COME TO BE?

DR. TROUNSON: THIS IS ONE OF THE BEST GROUPS, ONE OF THE BEST GENETIC ENGINEERING GROUPS IN THE COUNTRY, MAYBE THE WORLD. THEIR DATA IN VITRO, IN MY VIEW AND IN STAFF'S VIEW, IS REALLY QUITE SUPPORTIVE OF BEING ABLE TO GET THERE. OF COURSE, YOU DON'T REALLY KNOW UNTIL YOU ACTUALLY GET INTO THE PATIENTS. AND I THINK THAT'S WHERE THE CONCERNS OF THE REVIEWERS WERE, WHETHER THE IN VIVO CONVERSION WILL DO AS WELL.

SO I THINK IT'S RIGHT AT THE TOP OF WHAT MOST PEOPLE ARE SAYING IS APPROPRIATE. SO I THINK IT'S THERE. BUT, OF COURSE, WE'LL ONLY KNOW WHEN WE HIT THOSE CLINICAL TRIALS. AND THIS IS SUCH A TERRIBLE DISEASE. IF WE COULD ACTUALLY GET THERE WOULD BE WONDERFUL, AND SO WE'RE SUPPORTIVE FOR THAT POINT OF VIEW.

MR. SHEEHY: ARE WE READY FOR ROLL CALL ON THE MOTION? MARIA.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: MARCY FEIT. MICHAEL GOLDBERG.

MR. GOLDBERG: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.
MR. JUELSGAARD: YES.
MS. BONNEVILLE: FRANCISCO PRIETO.
DR. PRIETO: YES.
MS. BONNEVILLE: ROBERT QUINT.
DR. QUINT: YES.
MS. BONNEVILLE: AL ROWLETT.
MR. ROWLETT: YES.
MS. BONNEVILLE: JOAN SAMUELSON.
MS. SAMUELSON: ABSTAIN.
MS. BONNEVILLE: JEFF SHEEHY.
MR. SHEEHY: YES.
MS. BONNEVILLE: OS STEWARD. JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MR. HARRISON: MOTION CARRIES.
MR. SHEEHY: NOW ARE THERE ADDITIONAL MOTIONS TO MOVE A GRANT IN THIS CATEGORY TO TIER I? SENATOR TORRES.
MR. TORRES: YES. THE SECOND RECOMMENDATION MADE BY STAFF, WHICH IS TR4-06831.

MR. SHEEHY: DO WE HAVE SECOND?

DR. PRIETO: SECOND.

MR. SHEEHY: DR. PRIETO SECONDS.

ANY DISCUSSION? AGAIN, WE HAD THE EXCELLENT PRESENTATION BY DR. OLSON AND ALSO NOTED THAT ONE OF THE ISSUES WAS SOMEWHAT ADDRESSED IN THE NEW PUBLICATION THAT SUGGESTS THAT THEY HAVE A GREATER POSSIBILITY OF SUCCESS.

IS THERE ANY PUBLIC COMMENT? I'M SORRY, ALAN.

DR. TROUNSON: THAT'S ALL RIGHT, JEFF. THIS IS, OF COURSE, ONE OF THOSE ORPHAN DISEASES. IT'S A METABOLIC DISORDER THAT COULD BE REPAIRED WITH CONVERSION OF 4 PERCENT OF THE CELLS IN THE LIVER. SO IT'S A RELATIVELY LOW BAR COMPARED TO WHAT WE HAD TO ACHIEVE WITH WHAT'S NEEDED TO BE ACHIEVED WITH THE HEMATOPOIETIC STEM CELLS FOR THE OTHER DISEASES.

SO THERE HAS ALSO BEEN SOME PRETTY MAJOR ADVANCES MORE RECENTLY, SOME OF WHICH I REPORTED TO YOU JUST OVER THE LAST MONTH, THAT THEY'RE GETTING -- THEY'RE ABLE TO NOW DEVELOP HUMAN LIVER THROUGH TO FUNCTIONAL LIVER IN MICE. AND SO THIS WAS A BIG STEP FORWARD. AND FOR THAT REASON I PERSONALLY SORT OF CHANGED AND BECAME SUPPORTIVE

BECAUSE I THINK IF YOU CAN ACTUALLY DEVELOP THAT LIVER EFFECTIVELY, AND THERE'S NO REASON WHY THEY COULDN'T USE THE SAME TECHNOLOGY, THE CO-CULTURE TECHNOLOGY AS THE JAPANESE SCIENTISTS DID IN THEIR PAPER, THEN I THINK YOU CAN GET THAT 4 PERCENT EFFECTIVE. AND THESE KIDS WON'T SURVIVE LONGER THAN FIVE YEARS IF THEY DON'T GET IT.

SO IT'S A SMALL POPULATION, BUT A SIGNIFICANT ONE AND AN ORPHAN DISEASE THAT WE FEEL, AGAIN, IS ONE OF THOSE CONDITIONS THAT YOU MIGHT BE ABLE TO MOVE QUITE QUICKLY ON AND EFFECTIVELY ON, AND THAT'S WHY WE WERE SUPPORTIVE, REALLY SO SUPPORTIVE OF THIS PROJECT.

MR. SHEEHY: THANK YOU, DR. TROUNSON. FURTHER COMMENTS, BOARD? STAFF? ON THE PHONE? PUBLIC COMMENT ON GRANT 6831? WE HAVE SOME FOLKS HERE. IF ANYONE AT THE SITES HAS FOLKS, PLEASE LET ME KNOW. PLEASE INTRODUCE YOURSELF FOR OUR TRANSCRIBER.

DR. LIPSHUTZ: I'M GERRY LIPSHUTZ. I'M THE LEAD INVESTIGATOR ON THE STUDY YOU'RE PRESENTLY SPEAKING ABOUT, AND WE APPRECIATE THE OPPORTUNITY TO PROVIDE COMMENT ON THIS PROPOSAL THAT ADDRESSES AN UNMET NEED.

IN AFFLICTED NEONATES AND CHILDREN, ONE

COMMON THEME IS THE LACK OF THERAPIES ASIDE FROM LIVER TRANSPLANTATION, WHICH ARE PARTICULARLY RISKY AND COMPLICATED IN NEONATES AND CHILDREN. AS A GROUP, THE INCIDENCE OF NEONATAL LIVER DISEASES IS ONE IN 2500 LIVE BIRTHS IN THIS COUNTRY. IT ACCOUNTS FOR 15,000 HOSPITALIZATIONS EACH YEAR. THESE PATIENTS ACCOUNT FOR A SIGNIFICANT FRACTION OF CHILDREN WHO GET LIVER TRANSPLANTS. ABOUT 10 PERCENT AND THE VAST MAJORITY ARE FOR METABOLIC LIVER DISEASE WITH 560 CHILDREN UNDERGOING A LIVER TRANSPLANT IN 2010 IN THIS COUNTRY.

THIS IS A WIDERANGING CATEGORY AND INCLUDES DEFECTS IN AMINO ACID METABOLISM SUCH AS MAPLE SYRUP URINE DISEASE, TYROSINEMIA, PROPRIONIC ACIDEMIA, METHYLMALONIC ACIDEMIA. IT INCLUDES THE UREA CYCLE DEFECTS, INCLUDING OTC DEFICIENCY AND ARGINASE DEFICIENCY, THE ACTUAL DISEASE WE'RE ATTEMPTING TO TREAT WITH THIS GRANT. IT ALSO INCLUDES DEFECTS IN GLYCOGEN METABOLISM AND GLYCOGEN STORAGE DISORDERS. THESE LIMITED TREATMENTS TODAY ARE ONEROUS, POOR TASTING, AND AT BEST ONLY PARTIALLY EFFECTIVE. A COMMON THEME TO THESE, INCLUDING THE UREA CYCLE DISORDERS, IS THAT THEY HAVE METABOLIC DECOMPENSATION.

WHILE CHILDREN AWAIT A TRANSPLANT, SOME

WILL HAVE PERIODS WHERE THEY ARE DOING REASONABLY WELL AND THEN THEY'LL HAVE SOMETHING THAT TRIPS THEM OVER THE EDGE WHERE THEY GO INTO A CATABOLIC STATE AND START PRODUCING HIGHLY TOXIC BY-PRODUCTS. THEY GET ELEVATED AMMONIAS, CONFUSION, AND BRAIN SWELLING. IN THE CASE OF THE UREA CYCLE DISORDERS, THE FOCUS OF THIS PROPOSAL, THIS TYPICALLY OCCURS WITH ILLNESSES LIKE A COMMON COLD THAT CAN BE LIFE THREATENING, SUCH AS THEY GO TO THE ICU AND HAVE AN EMERGENCY DIALYSIS TO REMOVE TOXIC METABOLITES. IF THEY SURVIVE, MANY WILL HAVE BRAIN INJURIES AND DEVELOPMENTAL DELAYS.

THERE'S AN URGENT NEED FOR IMPROVED THERAPIES, AND WE REQUEST TODAY THAT YOU PLEASE CONSIDER OUR PROPOSAL. I'M HERE TODAY TO ASK YOU TO PLEASE CONSIDER FUNDING THIS AWARD TO DEVELOP NEW THERAPIES FOR CHILDREN AFFLICTED WITH INHERITED LIVER DISEASE AND FOR THEIR PARENTS WHO FACE THEIR OWN LIFETIME OF WORRY. THANK YOU FOR YOUR CONSIDERATION.

MS. DE LEON: HELLO. MY NAME IS ROBIN DE LEON, AND I HAVE OTC DEFICIENCY AS WELL AS MY DAUGHTER. I HAVE LOST TWO SONS TO OTC, ONE FOLLOWING HIS LIVER TRANSPLANT. AND THERE IS NOTHING WORSE THAN HAVING TO BURY YOUR CHILD.

LIVING WITH OTC IS HARD. I LIVE BY THE CLOCK, ADMINISTERING MEDS TO MYSELF AND MY DAUGHTER 11 TIMES A DAY, COUNTING PROTEIN, COUNTING CALORIES, COUNTING FLUID INTAKE, AVOIDING STRESSFUL SITUATIONS WHICH CAN CAUSE OUR AMMONIA LEVELS TO RISE.

I HAVE FOUND THAT OTC CAN SHOW ITS UGLY FACE AT ANY TIME. ONE MINUTE WE'RE OKAY AND THE NEXT MINUTE WE'RE NOT. WE HAVE TO AVOID BEING IN THE HEAT WHICH MAKES US SICK. ONE OF THE HARDEST THINGS FOR ME IS MY LITTLE GIRL. I HAVE TO HOME SCHOOL HER DUE TO GERMS. I DID HAVE HER IN THE PUBLIC SCHOOL AT ONE TIME, AND SHE WAS CATCHING EVERYTHING THAT CAME AROUND, WHICH PUT HER IN THE HOSPITAL. I CAN'T EVEN COUNT THE TIMES SHE'S BEEN HOSPITALIZED. SHE DOESN'T HAVE ANY FRIENDS, WHICH BREAKS MY HEART, SINCE THERE ISN'T ANY SOCIALIZING FOR HER. SHE'S A CHILD AND SHOULD BE OUT HAVING FUN WITH HER FRIENDS. IT'S VERY SAD.

WE FEEL SO ISOLATED AT TIMES. SHE CAN'T DO SPORTS BECAUSE OF THE PROTEIN THAT SHE CAN'T HAVE SO SHE CAN'T BUILD MUSCLE. SHE HAS LEARNING DISABILITIES AS WELL, SO AS I. SO MANY TIMES I BECOME CLOUDY WHERE I CAN'T CONCENTRATE OR FOCUS AND I LOSE TRACK OF TIME SINCE MY LEVELS FLUCTUATE ALL DAY LONG. NOT ONLY DOES THIS AFFECT OUR LIVER, BUT

OUR BRAINS AS WELL.

I ALONG WITH MY DAUGHTER HAVE PARTICIPATED IN MANY STUDIES FOR UREA CYCLE DISORDER AND IT'S IMPORTANT TO KNOW SO MANY THINGS ABOUT US THAT DOCTORS DIDN'T KNOW SINCE THEY DO PROVIDE DIFFERENT TESTS THAT AREN'T NORMALLY DONE. MY HOPE IS THAT ONE DAY WE FIND A CURE. I CAN'T STRESS ENOUGH THE IMPORTANCE THAT RESEARCH AND STUDIES MEAN TO US BECAUSE WE GET LEFT BEHIND BECAUSE WE ARE A RARE BREED AND DOCTORS NEED TO BE MORE EDUCATED ABOUT US.

THANK YOU FOR ALLOWING ME TO HAVE THE OPPORTUNITY TO TALK TO YOU.

MR. SHEEHY: THANK YOU.

MS. SONTAG: MY NAME IS AMANDA SONTAG. MY ANTHONY HAS UREA CYCLE DISORDER, SPECIFICALLY OTC. HE WAS DIAGNOSED WHEN HE WAS 22 MONTHS OLD AND NEXT MONTH HE WILL BE 16. HE'S A SOPHOMORE IN HIGH SCHOOL, PLAYS BASEBALL, HAS GOOD GRADES, AND ASPIRATIONS TO GO INTO THE MEDICAL FIELD. HE'S BEEN HOSPITALIZED MORE TIMES THAN I CAN COUNT, INCLUDING BEING AIRLIFTED FROM CHOC ADMISSION TO UCLA IN 2010, THE WORST TIME IN OUR LIVES. HE WAS ALMOST IN A COMA WITH ENCEPHALOPATHY SECONDARY TO HYPERAMMONEMIA. WE'VE BEEN VERY FORTUNATE THAT HE'S NOT SUFFERED ANY PERMANENT DISABILITY OR BRAIN

DAMAGE.

MANAGING A CHILD WITH UREA CYCLE DISORDER MEANS NEVER ENDING MONITORING OF EVERYTHING THAT GOES IN HIS MOUTH, MAKING SURE ALL MEDICINE, MEDICAL FORMULA AMINO ACIDS ARE CONSUMED EVERY DAY. HAVING BLOOD TESTS TAKEN EVERY FEW MONTHS ARE A PART OF HIS LIFE FOREVER.

THE WORRY WHEN HE IS AROUND OTHER PEOPLE WITH A COLD OR A FLU IS ENORMOUS SINCE THIS CAN BECOME METABOLICALLY UNSTABLE VERY EASILY AND USUALLY RESULTS IN HOSPITALIZATION. BY ALL STANDARDS ANTHONY WOULD BE CONSIDERED VERY HIGH LEVEL AND VERY LUCKY.

BY THE GOOD FORTUNE OF HAVING GREAT DOCTORS AT UCLA, I'M HERE TO POINT OUT THAT THERE ARE MANY OTHER FAMILIES WHICH THE DISORDER WAS DISCOVERED IN A SIMILAR MANNER HAVE HAD FAR LESS FAVORABLE OUTCOMES. THEY LIVE WITH SEVERELY DISABLED CHILDREN OR HAVE LOST THEM COMPLETELY DUE TO THIS TERRIBLE DISORDER.

MANY ADULTS AND CHILDREN FIND THE MEDICAL FORMULA INTOLERABLE TO SWALLOW AND REQUIRE G TUBE OR NG TUBE PLACEMENT TO IMPROVE METABOLIC STABILITY.

WHEN YOU BECOME ON A FIRST NAME BASIS WITH ER AND ICU NURSES AND DOCTORS, YOU'VE SEEN EACH

OTHER TOO MUCH. UNFORTUNATELY THIS IS THE LIFE OF LIVING WITH UREA CYCLE DISORDER. WE HAVE UNCERTAINTY EVERY DAY AND THE WORRY WILL NEVER END.

OUR FAMILY HAS ALWAYS BEEN SUPPORTIVE OF RESEARCH FOR UREA CYCLE DISORDERS. BOTH MY SON AND MYSELF, BEING A CARRIER, HAVE BEEN PARTICIPATING IN LONGITUDINAL STUDIES FOR SEVERAL YEARS NOW. ANTHONY JUST FINISHED PARTICIPATING IN THE DRUG TRIAL WHICH HE STARTED IN PHASE I FOR SEVERAL YEARS CALLED HPN 100, WHICH RECENTLY BECAME FDA APPROVED AND IS NOW CALLED RAVICTI.

WE KNOW THE POTENTIAL FOR A CURE FOR ANTHONY AS WELL AS COUNTLESS OTHERS LIE IN STEM CELL RESEARCH. ENZYME REPLACEMENT THERAPY IS NOT A FEASIBLE CURE FOR OTC SINCE IT COULD ONLY WORK IN THE LIVER MITOCHONDRION. IF DR. LIPSHUTZ IS SUCCESSFUL IN HIS APPROACH OF STEM CELL THERAPY, THIS WOULD TAKE OFF THE BURDEN OF CONSIDERING LIVER TRANSPLANT OR LIFELONG LOW PROTEIN DIET MANAGEMENT, EXTRAORDINARILY EXPENSIVE MEDICATION, AND UNCERTAINTY.

EVEN THOUGH THIS PROJECT IS FOCUSED ON ARGINASE DEFICIENCY, IT IS APPLICABLE FOR OTC AS WELL. WHEN ANTHONY WAS DIAGNOSED, MY HUSBAND AND I HAD A DREAM OF MAYBE THERE WILL BE A CURE WHEN HE'S

IN HIGH SCHOOL. NOW OUR DREAM IS FOR MAYBE WHEN HE GRADUATES COLLEGE. YOU CAN HELP THIS DREAM COME TRUE BY FUNDING THIS PROJECT. THANK YOU.

MR. SHEEHY: THANK YOU.

MS. WILSON: I WOULD LIKE TO THANK THE BOARD FOR YOUR CONSIDERATION OF TR4-06831. UREA CYCLE PATIENTS ARE TORTURED BY THE INABILITY TO EAT NORMALLY OR NOTHING BUT CRUMBS MADE UP OF HORRIBLE TASTING FOODS, SUBJECTED TO MEDICATIONS AND TREATMENTS THAT MOST OF US COULD NEVER ENDURE. THEY ENDURE MEDICATIONS THAT BURN GOING DOWN, BURN COMING BACK UP, AND OFTENTIMES DAMAGE THE ESOPHAGUS BEYOND REPAIR. ONE MEDICATION CAN COST THOUSANDS A MONTH, AND THESE PATIENTS TAKE MANY. G TUBES, DIARRHEA, SEIZURES, WHEELCHAIRS, LEG BRACES, WALKERS, BLOOD DRAWS, HOSPITALIZATIONS, EXTREME PRAYING, AND RETARDATION ARE OFTEN THE WORLD THAT THESE PEOPLE ARE CAUGHT IN.

IN 1995 OUR SON WAS DIAGNOSED WITH ARGINASE DEFICIENCY. IMMEDIATELY HE STARTED THROWING UP ON A BAD DAY 15 TIMES A DAY. AMMONIA LEVELS WOULD GO OUT OF CONTROL. JACKSON'S ACTIVITIES WERE SEVERELY RESTRICTED BECAUSE OF HIS VOMITING AND FALLING. DAYS AND NIGHTS WERE SPENT IN BED OR IN HOSPITALS, UNABLE TO STOP HIS VOMITING OR

SEIZURES. WITH ALL THIS, WE WERE COMMITTED TO LIVING A NORMAL LIFE. BUT WITH CONSTANT VOMITING, IT WAS DIFFICULT FOR US AND VERY UNCOMFORTABLE FOR OTHERS. SO WE WOULD TAKE ALONG A BUCKET AND A TOWEL. ON EACH OF OUR OUTINGS, JACKSON, BY THE WAY, THREW UP 125 DAYS 15 TIMES A DAY. IMAGINE IF THAT WERE YOUR CHILD. SLEEPLESS NIGHTS, SPENDING HOURS MAKING NECESSARY MEDICATIONS, DRINKS, AND HORRIBLY NASTY FORMULAS, DEVASTATED WATCHING DOCTORS INJECT BOTOX THROUGHOUT THE CALVES OF HIS LEGS WHILE THEY HELD HIM DOWN SCREAMING.

LIFE SEEMED IMPOSSIBLE FOR US. WE WENT ON BLAMING EACH OTHER, BLAMING THE DOCTORS IN SOME INSTANCES, AND WORST OF ALL WE BLAMED GOD. JACKSON IS NOT A GOOD EXAMPLE OF SOMEONE THAT REALLY WOULD BENEFIT GREATLY FROM YOUR GENEROSITY. THE PATIENTS THAT WOULD REALLY BENEFIT FROM YOUR GIFT AND YOUR CONSIDERATION EITHER PHYSICALLY CAN'T MAKE THIS TRIP DOWN HERE AND SEEK YOUR SUPPORT OR THEY CAN'T AFFORD IT. THE YEARS OF EXPENSIVE DRUGS AND TREATMENT HAVE LEFT THEIR FINANCES DEPLETED AND ALL BUT GONE. AND IN SOME CASES THEY'VE JUST SIMPLY GIVEN UP AND DON'T WANT TO COME.

WE'RE HERE FOR THE CHILDREN THAT CAN'T DEFEND THEMSELVES OR SPEAK UP AND TELL YOU WHY YOU

SHOULD MAKE A FAVORABLE DECISION TO SUPPORT DR. LIPSHUTZ AND THE UCLA RESEARCH TEAM FOR UREA CYCLE. I WAS GOING TO SHOW YOU A BUCKET LIKE THE ONE WE USED TO USE WITH THE TOWEL AND LET YOU THINK IF THAT WAS YOUR CHILD HOW IT WOULD FEEL TO TAKE AROUND EVERYWHERE YOU WENT. IT'S PRETTY HARD TO DO, AND JUST VISUALIZE YOUR CHILD'S PICTURE ON THAT. I HAVE SIX BUCKETS OF THE PEOPLE THAT WE KNOW THAT USE THEM WITH THEIR PICTURES ON THERE. YOU'LL JUST HAVE TO KIND OF IMAGINE WHAT THAT WOULD LOOK LIKE.

YOU CAN GRANT DR. LIPSHUTZ ABILITY TO CURE UREA CYCLE AND OTHER DEFECTS. EACH OF YOU HERE TODAY CAN SAVE OTHER PATIENTS AND PARENTS FROM THE PAIN OF ABSOLUTE DEVASTATION OF LIVING WITH UREA CYCLE. YOU CAN BE THE ONE THAT MAKES THE DIFFERENCE. WHAT IF IT WERE YOUR CHILD? WHAT WOULD YOUR DECISION BE? AND I WONDER WHAT DUANE ROTH WOULD THINK AND DECIDE ON. THANK YOU FOR THIS. MY NAME IS LEATHY WILSON.

MR. SHEEHY: THANK YOU.

MS. WILSON: THANK YOU VERY, VERY MUCH, SIR.

MS. FUKUDA: MY NAME IS JEAN FUKUDA, AND I'M JACKSON FUKUDA'S MOTHER WHO HAS ARGINASE DEFICIENCY. AND LEATHY JUST TOUCHED UPON ALL THE

HARDSHIP THAT WE WENT THROUGH WHEN HE WAS FIRST DISCOVERED WITH ARGINASE DEFICIENCY. AND I JUST WANT TO FAST FORWARD THIS TEN YEARS. NOW JACKSON IS 22 YEARS OLD. AND I JUST WANT TO TOUCH LITTLE BIT ON THE EXPENSE OF JUST KEEPING HIM HEALTHY. THIS IS EVERYDAY LIFE.

EVERY DAY HE HAVE TO HAVE THIS METABOLIC DRINK, WHICH IS THIS CONTAINER. THIS IS $255, THREE AND A HALF DAYS SUPPLY, PLUS PROFREE, WHICH IS $55 A DAY -- I'M SORRY -- $55 A CAN. IT'S THREE-AND-A-HALF-DAY SUPPLY. AND WE MIX THIS WITH LEMON JUICE, AND THE ONE-MONTH SUPPLY OF THIS PRODUCT IS $2,840. ON TOP OF THAT, THIS IS HOW MUCH HE HAVE TO TAKE EVERY DAY, AND IT'S MIXED WITH LEMON JUICE. IT MAKES ABOUT 20 FLUID OUNCES OF DRINK. AND CAN YOU IMAGINE TEN YEARS OUR KID, ME TRYING TO HAVE HIM TAKE 20 OUNCES OF THIS YUCKY STUFF. ON TOP OF THAT, HE HAVE TO TAKE 20 CAPSULES EVERY DAY OF THIS SODIUM BENZOATE. AND YOU COULD IMAGINE TEN YEARS, TRYING TO TAKE 20 CAPSULES EVERY DAY.

AND ALSO WE PACKED THIS OURSELVES. THIS IS HAND PACKED WITH VEGETARIAN CAPSULES BECAUSE THIS SODIUM BENZOATE IS REALLY BAD TASTING. IT BURNS YOUR THROAT AS HE TAKES IT. SO THIS IS WHAT WE DO. WE PACK THIS. THIS IS ONLY ABOUT TWO DAY'S SUPPLY,

SO YOU CAN IMAGINE WE SPEND A LOT OF TIME PACKING THESE CAPSULES.

AND ALSO JACKSON'S INSURANCE RUNS ABOUT $741 A MONTH, AND IT'S BEEN GOING UP 15 PERCENT EVERY MONTH. AND TO MAINTAIN HIS HEALTH, EVERY SIX MONTHS HE GETS COMPLETE BLOOD TEST ON TOP OF MAINTAINING DETAILED ACCOUNTS OF EVERYTHING THAT HE EATS EVERY DAY. AND AS A RESULT OF THIS TEST, PLUS THIS, WE DETERMINE HOW MUCH PROTEIN AND HOW MUCH CALORIES HE COULD TAKE EVERY DAY, AND THIS GOES ON EVERY DAY. JUST TO KEEP JACKSON'S HEALTH, WE HAVE SEVERAL TEAMS OF DOCTORS WORKING WITH US, METABOLIC TEAM, ORTHOPEDIC TEAM TO MONITOR HIS OSTEOPOROSIS AND SPASTICITY, ALSO JUST TO MONITOR HIS GLAUCOMA NOT KNOWING HIGH CONCENTRATION OF ARGININE BUILDUP ON HIS VISION. SO WE'RE NOT SURE ABOUT THAT. AND ALSO DENTIST TO MAKE SURE THAT HIS TEETH IS NOT AFFECTED BY LOWER INTAKE OF PROTEIN. AND THIS IS HIS LIFE FOR REST OF HIS LIFE. THIS IS OUR REGIMENT WE HAVE TO DO EVERY DAY TO KEEP HIS HEALTH.

SO WE'RE JUST REALLY HOPING THAT THIS WILL BE APPROVED. AND NOT ONLY THAT, BUT THE NEW BONE SCREENING TEST IS NOW INCLUDING UREA CYCLE DEFECT, SO THERE'S GOING TO BE A LOT MORE BABIES THAT'S GOING TO BE DISCOVERED WITH THIS DISEASE. SO IT'S

SO IMPERATIVE THAT THESE BABIES GET HELP THAT THEY NEED SO THEY COULD HAVE NORMAL LIFE. AND NOW YOU GET TO MEET MY SON JACKSON.

MR. SHEEHY: THANK YOU.

MR. FUKUDA: I'M JACKSON FUKUDA. I'M THE SON OF JEAN FUKUDA AND LEATHY WILSON. I'M 22 YEARS OLD AND I WILL SOON BE GRADUATING FROM CALIFORNIA BAPTIST UNIVERSITY WITH A DEGREE IN GRAPHIC DESIGN.

AS PREVIOUS SPEAKERS HAVE OUTLINED, THE MEDICATION IS PRIMARILY THE WORST PART OF IT. BOTOX SPECIFICALLY, EVEN THOUGH I'VE HAD IT MANY YEARS AGO; BUT AS THEY HAVE OUTLINED, THE PAIN WAS EXTREME AND ACTUALLY THEY HAD TO HOLD ME DOWN IN CASE I WOULD HURT MYSELF OR OTHERS.

WE'VE TRIED OTHER MEDICATIONS THAT HAVE LEFT DAMAGE TO THE ESOPHAGUS AND STOMACH OF MYSELF AND OTHER PATIENTS. SO FAR THE ONLY ONE THAT SEEMS TO WORK IS SODIUM BENZOATE THAT IS TAKEN WITH CAPSULES.

THERE ARE OTHER RESTRICTIONS SUCH AS EXERCISES, AND PHYSICAL ACTIVITIES CAN BE LIMITED DUE TO HOW THE BODY DEVELOPS IF YOU DON'T HAVE ENOUGH MUSCLE OR THE BONES ARE WEAK OR SOMETHING THAT ALLOWS US NOT TO BE ABLE TO DO MUCH. I WAS A LUCKY CASE, AND I'M NOT A GOOD EXAMPLE OF SOMEONE

WHO NEEDS ALL THIS FUNDING BECAUSE I PRETTY MUCH HIT THE PLATEAU OF WELLNESS FOR SOMEONE WITH THIS DISORDER. THE PEOPLE WHO NEED IT ARE YOUNGER OR THEY'RE NOT EVEN BORN YET AND WHO WILL HAVE THIS DISORDER. THERE MAY BE PEOPLE WHO ARE SUFFERING WITH IT CURRENTLY AND THEIR LIVES ARE A LOT WORSE THAN MINE. I'M THE ONLY ONE SO FAR THAT HAS MADE IT THIS FAR. NO ONE ELSE HAS. AND I'M GOING TO HOPE THAT I'M NOT GOING TO BE THE LAST ONE THAT MAKES IT THIS FAR.

MR. SHEEHY: THANK YOU. I JUST WANT TO THANK ALL OF YOU FOR COMING AND SHARING YOUR STORIES AND YOUR SACRIFICES. I KNOW, SPEAKING FOR MY FELLOW BOARD MEMBERS, WE'RE ALL MOVED DEEPLY AND OUR HEARTS GO OUT TO YOU.

ARE THERE FURTHER COMMENTS FROM ANY OTHER SITE?

DR. DULIEGE: I JUST WANT TO SECOND WHAT YOU JUST SAID ON BEHALF OF ALL OF US, INDEED, THE FIRST TIME FROM YOU AND WANTING TO THANK YOU FOR YOUR COURAGE TO SHARING YOUR STORIES WITH US.

MR. SHEEHY: I THINK WE'RE READY FOR A ROLL CALL. AND THE MOTION IS TO APPROVE THIS GRANT.

DR. DULIEGE: SECOND.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.
MS. BONNEVILLE: MARCY FEIT. MICHAEL GOLDBERG.
MR. GOLDBERG: YES.
MS. BONNEVILLE: STEVE JUELSGAARD.
MR. JUELSGAARD: YES.
MS. BONNEVILLE: FRANCISCO PRIETO.
DR. PRIETO: AYE.
MS. BONNEVILLE: ROBERT QUINT.
DR. QUINT: YES.
MS. BONNEVILLE: AL ROWLETT.
MR. ROWLETT: YES.
MS. BONNEVILLE: JOAN SAMUELSON.
MS. SAMUELSON: ABSTAIN.
MS. BONNEVILLE: JEFF SHEEHY.
MR. SHEEHY: YES.
MS. BONNEVILLE: OS STEWARD. JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MS. BONNEVILLE: MOTION CARRIES.
MR. SHEEHY: AGAIN, THANK YOU FOR COMING

TODAY.

DO WE HAVE ADDITIONAL MOTIONS TO MOVE ANYTHING IN TIER II OR TIER III INTO THE FUNDABLE CATEGORY?

CHAIRMAN THOMAS: JAMES, THE APPROPRIATE QUESTION AT THIS POINT IS DO WE HAVE MOTIONS TO MOVE ANYTHING FROM TIER III; IS THAT CORRECT?

MR. GOLDBERG: YES, WE HAVE A MOTION.

MR. SHEEHY: ARE YOU MAKING THE MOTION?

MR. GOLDBERG: YES.

MR. SHEEHY: TO MOVE WHICH GRANT?

MR. GOLDBERG: TR4-06888, SPINAL CORD INJURY.

MR. SHEEHY: I DON'T THINK WE'RE IN THAT CATEGORY YET. THAT'S A DEVELOPMENT CANDIDATE.

MR. GOLDBERG: MY APOLOGIES.

MR. SHEEHY: I THINK WHAT WE'RE LOOKING AT IS ARE THERE ANY MOTIONS TO MOVE ANYTHING REMAINING INTO TIER I?

DR. DULIEGE: JEFF, IT'S NOT YET A MOTION, BUT THINGS WENT VERY QUICKLY, AND I HAD A QUESTION REGARDING 06666. IS THAT ACCEPTABLE NOW TO ASK A QUESTION?

MR. SHEEHY: THAT'S IN THE OTHER CATEGORY. WE HAVE TWO CATEGORIES OF GRANTS. THAT'S HERE. I'M

SORRY.

DR. DULIEGE: I THINK IT'S IN THIS CATEGORY. THAT WAS THE FIRST ONE. SO IT'S 6666. MAY I ASK A QUESTION?

MR. SHEEHY: SURE.

DR. DULIEGE: THE RECOMMENDATION WAS NOT TO FUND, AND ONE OF THE CONSIDERATIONS WAS THAT THERE WERE ALREADY SIMILAR APPROACHES THAT WERE FUNDED. UNDERSTANDING THAT WE HAVE TO BE PARTICULARLY CAREFUL BECAUSE THERE IS MORE AND MORE LIMITED MONEY TO SPEND, I'D LIKE TO HAVE A LITTLE BIT MORE COMMENTS ON THAT FROM THE DISEASE WORKING TEAM BECAUSE, INDEED, THERE HAVE BEEN OTHER MOTIONS THAT HAVE BEEN RECOMMENDED FOR FUNDING AND YET THERE WERE SEVERAL OTHER APPROACHES. SO JUST VERIFICATION ON THIS. THANK YOU.

DR. VUORI: CAN I QUICKLY FOLLOW UP ON THAT? SO IF THE STAFF MADE THE COMPARE AND CONTRAST THIS TR4-06666 IN THE DCF CATEGORY TO THE DC CATEGORY, APPLICATION TR4-06648, THAT WAS MOVED WITH THE CONDITION TO TIER I. HOW DO THESE TWO APPROACHES COMPARE AND CONTRAST EACH OTHER?

DR. TROUNSON: I THINK DR. OLSON IS GOING TO RESPOND TO THAT.

DR. STEFFEN: I'M DR. STEFFEN. WHAT I

THINK WE'RE GOING TO DO IS GIVE YOU INDIVIDUAL PRESENTATION ON THE APPLICATION 6666, WHICH IS THE ONE YOU ASKED ABOUT, AND THEN ANOTHER SCIENCE OFFICER IS GOING TO ADDRESS THE OTHER APPLICATION, AND THEN DR. OLSON THE COMPARISON TO THE DISEASE TEAM AWARD.

SO APPLICATION 6666 IS TO DEVELOP A FEASIBILITY ASSESSMENT TO INVESTIGATE DEVELOPING HUMAN EMBRYONIC STEM CELL OR HUMAN INDUCED PLURIPOTENT STEM CELL-DERIVED PHOTORECEPTOR CELLS AS POTENTIAL THERAPY FOR PATIENTS WITH INHERITED RETINAL DISORDERS. LIKE THE MORE COMMON MACULAR DEGENERATION, THEY CAN BOTH CAUSE BLINDNESS, BUT THIS PRIMARILY AFFECTS A YOUNGER POPULATION, SOME OF THESE IN CHILDREN AND SOME MOSTLY IN THEIR FOURTH DECADE OF LIFE.

SO THIS APPLICANT PROPOSES TO OPTIMIZE THE DIFFERENTIATION PROTOCOL TO ACHIEVE THE PHOTORECEPTORS, AND THEN SELECT THE MOST APPROPRIATE EITHER EMBRYONIC STEM CELL OR IPS CELL LINE AND THEN DEVELOP ENRICHMENT METHODS AND ASSAYS TO GET THAT ACTUAL PHOTORECEPTOR AND TEST THE OPTIMAL TRANSPLANTATION PARAMETERS IN TWO LABORATORY MODELS OF THE INHERITED RETINAL DEGENERATION, KIND OF THAT CLASSIC PRECLINICAL RESEARCH SPACE.

I'M GOING TO BE GO THROUGH THE REVIEWER COMMENTS. THEY WERE QUITE POSITIVE ABOUT THE STRUCTURE OF THE GRANT, THE OBJECTIVE OF THE PROPOSAL TO GENERATE THE PHOTORECEPTOR CELLS, SIGNIFICANT AND IMPORTANT IN FOCUSING ON THESE SPECIFIC PHOTORECEPTOR CELLS. THE MILESTONES WERE PRESENTED IN A LOGICAL FASHION FOR ACHIEVING THE PRECLINICAL PROOF OF CONCEPT IN A PRELIMINARY STATUS.

THE RATIONALE AND SIGNIFICANCE, THEY ARE USING A DIFFERENT APPROACH -- SORRY. IN GENE THERAPY FOR THESE INHERITED RETINAL DISORDERS, THE FIELD KNOWS THAT JUST A SMALL INCREMENTAL INCREASE OF PHOTORECEPTOR RESTORATION CAN MAKE A SIGNIFICANT CLINICAL DIFFERENCE. SO THE REVIEWERS COMMENTED THAT THEY FEEL COMFORTABLE ABOUT A CELL-BASED THERAPY APPROACH, KNOWING THAT A SMALL INCREMENTAL CHANGE CAN MAKE A CLINICAL BENEFIT.

THE RATIONALE TO PURSUE THE PHOTORECEPTOR CELL IS STRONG, AND A RELIABLE, WELL-CHARACTERIZED SOURCE OF RETINAL CELLS WOULD BE USEFUL TO THE FIELD FOR TISSUE REPLACEMENT PURPOSES.

THEY ALSO COMMENTED THAT THIS PARTICULAR PROJECT WOULD LIKELY INCREMENTALLY ADVANCE THE FIELD, BUT ACKNOWLEDGED THERE WERE NO TREATMENTS.

SO THAT IS A STEP IN THE RIGHT DIRECTION.

THE CHALLENGE FOR THIS AWARD WITH THE REVIEW GROUP WAS IN ITS FEASIBILITY AND DESIGN. AND WHILE THEY HAD SOME STRONG DATA SHOWING THAT INDUCTION OF THE CELLS COULD BE ACHIEVED FOR RETINAL MARKERS, THERE WAS A VERY LOW EFFICIENCY GOING TO THE FINAL PHOTORECEPTOR STAGE. AND A CHALLENGE FOR THAT PROJECT WILL BE TO INCREASE THE EFFICIENCY OF THE PROCESS AND ALSO IMPROVE THE STABILITY OF THAT FINAL CELL PHENOTYPE. WHEN THEY ACHIEVE THE PHOTORECEPTOR, THE CELL WAS MOVING AWAY FROM THAT FINAL DESIRED CELL TYPE. SO GETTING STABILITY OF THAT FINAL PRODUCT.

AND THEN THERE WAS A LOT OF DISCUSSION ABOUT THE OUTCOME MEASURES AND THE EXPERIMENTS AS THEY WERE DESIGNED TO ASSESS THE PROOF OF CONCEPT. AND THERE HAD BEEN A RECENT PAPER ABOUT A YEAR AGO SHOWING THAT THE PLANNED READOUT IN THE APPLICATION WOULD BE INADEQUATE FOR MEASUREMENT OF FUNCTIONAL BENEFIT. SO ANOTHER SCIENTIST HAD COME ALONG AND SAID THAT TISSUE INTEGRATION WAS ACTUALLY INSUFFICIENT TO PREDICT FUNCTIONAL BENEFIT AND SUGGESTED THAT MORE EXPERIMENTS BE DONE.

AND THEN OPTIMIZING THE DIFFERENTIATION FOR THE PHOTORECEPTOR CELLS WERE CONSIDERED A

SIGNIFICANT AMOUNT OF WORK CRITICAL TO THE SUCCESS OF THE PROJECT AND ACKNOWLEDGED AS A RISK.

SO THAT WAS REALLY THE CRUX OF THE DISCUSSION ON THE APPLICATION.

THE PI WAS CONSIDERED TO HAVE COME FROM A VERY PROMINENT LABORATORY THAT WORKS ON THE RETINA AND THESE CELL TYPES, WAS A NEWLY APPOINTED INVESTIGATOR IN 2001 AT THE APPLICANT INSTITUTION, AND SINCE THAT TIME PRODUCTIVITY HAS BEEN A LITTLE BIT MORE MODEST. AND THEN THE GRANTS REVIEW WAS CONCERNED THAT A NUMBER OF KEY PROJECT TEAM MEMBERS WERE LISTED AS TO BE HIRED. SO WITH A VERY CHALLENGING PROJECT AND A NUMBER OF STAFF NOT YET IN PLACE, IT WAS CONSIDERED A CHALLENGING THREE-YEAR TIMELINE.

THE INSTITUTION WAS RECOGNIZED TO HAVE ALL THE RESOURCES TO SUPPORT THE INDIVIDUAL, THE NECESSARY MATERIALS WERE IN PLACE, AND THE PI AND PREVIOUS MENTOR HOLD A KEY PATENT FOR THE APPROACH.

SO ON BALANCE, I THINK IT WAS THIS BALANCE BETWEEN THE RATIONALE AND SAYING APPROPRIATE TARGET, INTERESTING CANDIDATE, AND A VERY CHALLENGING TIME FRAME, VERY CHALLENGING TEAM AND INVESTIGATOR. THAT'S GOING TO END THE PRESENTATION ON THIS APPLICATION. DID YOU HAVE ANY MORE QUESTIONS?

DR. OLSON: WHAT I'M GOING TO DO -- IF YOU WANT TO HEAR MORE ABOUT THE OTHER APPLICATION, DR. ARI ABO WILL TALK ABOUT IT. I WANT TO SPECIFICALLY ADDRESS DR. VUORI'S QUESTION, WHICH WAS COMPARE AND CONTRAST.

SO AS YOU JUST HEARD FROM DR. STEFFEN, THAT APPLICATION IS TARGETING THE GENERATION OF FULLY DIFFERENTIATED PHOTORECEPTOR CELLS AS OPPOSED TO THE OTHER APPLICATIONS, WHICH IN ONE FORM OR ANOTHER ARE TARGETING THE GENERATION OF PROGENITOR CELLS. SO IT IS A CONCERN IN THE FIELD. TYPICALLY MATURE CELLS DON'T ENGRAFT. I THINK YOU JUST HEARD WHAT DR. STEFFEN SAID ABOUT THE CHALLENGES IN GETTING FULLY PHOTORECEPTOR CELLS. IT'S ALSO IN THE RETINA THE DIRECTIONAL PART OF IT. SO PUTTING IT IN A THREE-DIMENSIONAL SHEET ALONG WITH ITS SUPPORTING CELLS, THE RPE, WAS CONSIDERED TO BE A WAY OF ENSURING THAT YOU HAVE A -- MORE ENSURING THAT YOU HAVE A FUNCTIONAL STRUCTURE. SO IT'S PROGENITOR CELLS VERSUS FULLY DIFFERENTIATED CELLS. IT'S A STRUCTURAL ORGANIZATION TO THOSE CELLS THAT REPLICATES THAT THAT IS MORE LIKE THE EYE, AND IT'S THE SUPPORT CELLS.

SO THOSE ARE THE, I THINK, REASONS THAT, AT LEAST PART OF THE RATIONALE WHY STAFF MADE THE

RECOMMENDATION THEY DID WITH RESPECT TO THAT.

DOES THAT ANSWER YOUR QUESTION? AND THEN AS I SAY, DR. ABO IS HAPPY TO GO INTO MORE DETAIL ABOUT THE OTHER APPLICATION IF YOU WOULD LIKE.

DR. VUORI: THANK YOU.

DR. OLSON: THANK YOU.

MR. SHEEHY: ANY OTHER QUESTIONS OR COMMENTS?

DR. DULIEGE: GENERAL QUESTION, THEN, TO FINISH ON THE APPLICATION 6666. I UNDERSTAND WHAT WAS SAID AND I COMPLETELY AGREE WITH IT. WILL THERE BE OTHER APPLICATIONS THAT CAN IMPROVE HIS PROPOSAL AND COME BACK WITH IT OVER TIME?

DR. TROUNSON: THE ANSWER IS YES.

DR. STEFFEN: IN ADDITION TO THE PUBLIC SUMMARY THAT WE PREPARE, WE ALSO PROVIDE THE CONFIDENTIAL FEEDBACK. AND SO THE SPECIFICS OF THE RECOMMENDATIONS AROUND HOW THE INVESTIGATOR COULD IMPROVE THE EXPERIMENTAL DESIGN TO ADDRESS THE CONCERNS HAS BEEN PROVIDED CONFIDENTIALLY TO THE APPLICANT. AND I KNOW THE CIRM STAFF WOULD BE VERY HAPPY TO FOLLOW UP WITH SUCH AN APPLICANT OR ANY APPLICANT THAT WOULD LIKE TO DISCUSS THAT IN MORE DETAIL.

MR. SHEEHY: ANY OTHER BOARD OR STAFF

COMMENTS? THEN I THINK WE'RE AT THE POINT -- I THINK JAMES MAY HAVE TO HELP ME ON THE FRAMING -- FOR WHAT I WOULD CALL A GLOBAL RESOLUTION.

DR. BRYANT: SO IF THIS IS -- IF I MAKE A COMMENT ABOUT THE ENTIRE SCORED GROUP, DOES THAT PUT ME IN CONFLICT SINCE I'M IN CONFLICT FOR ONE OF THEM?

MR. HARRISON: YES. THAT POTENTIALLY POSES A PROBLEM.

DR. BRYANT: OKAY. THEN I WON'T SAY WHAT I WAS GOING TO SAY. SORRY.

MR. SHEEHY: ANY OTHER? SO I THINK WE'RE READY FOR A GLOBAL RESOLUTION, WHICH I BELIEVE THE FORM OF WHICH, AND I THINK IF YOU COULD RESTATE IT FOR ME BEFORE WE MAKE IT, BUT I THINK IT WOULD BE TO APPROVE ALL THE APPLICATIONS IN TIER I, INCLUDING THOSE THAT WE'VE MOVED IN THERE TODAY WITH THE ACCOMPANYING CONDITIONS, AND TO NOT APPROVE ALL THE OTHER REMAINING APPLICATIONS.

MR. HARRISON: JUST TO CLARIFY, THIS IS FOR DCF.

MR. SHEEHY: ONLY FOR DCF, THIS FIRST CATEGORY. DO I HAVE A MAKER OF THE MOTION WITHOUT CONFLICTS?

DR. JUELSGAARD: SO MOVED.

MR. SHEEHY: SO STEVE JUELSGAARD IS THE MAKER AND SENATOR TORRES IS THE SECOND.

NOW, ARE THERE ANY PUBLIC COMMENTS EITHER HERE OR IN ANY OF THE SITES ABOUT ANY APPLICATIONS IN THIS DCF CATEGORY? I BELIEVE WE HAVE ONE HERE. SO WE'LL HEAR THAT. AND I THINK, MICHAEL, YOU HAVE PEOPLE AT YOUR SITE. IF THERE'S ANY COMMENTS THERE, LET ME KNOW AFTER WE HEAR THE ONE HERE IN SAN FRANCISCO.

MS. SAMUELSON: I HAVE A QUESTION.

MS. THOMPSON: MY NAME IS LESLIE THOMPSON. I'M FROM THE UNIVERSITY OF CALIFORNIA IRVINE. I'M THE PI ON THE APPLICATION, THE NEXT APPLICATION DOWN, THAT HAS A SCORE OF 64 THAT IS IN TIER III, BUT IS VERY, VERY CLOSE TO THE PAYLINE. AND THANK YOU FOR THE OPPORTUNITY TO MAKE A FEW COMMENTS ABOUT THIS APPLICATION.

I WOULDN'T BE HERE IF I DIDN'T STRONGLY BELIEVE THAT THIS IS A VERY STRONG RESEARCH APPROACH AND A NEW STRATEGY TO TREAT HUNTINGTON'S DISEASE. AS EVERYONE HERE HAS HEARD ABOUT HUNTINGTON'S DISEASE, IT'S A DEVASTATING DISORDER, STRIKES INDIVIDUALS IN THE PRIME OF LIFE WHEN THEY HAVE YOUNG FAMILIES, THEY HAVE MAXIMUM EARNING POTENTIAL, STRIKES YOUNG ADULTS, CHILDREN. WE HAVE SEVERAL OF

THE FAMILY MEMBERS HERE. AND THIS REPRESENTS A NEW APPROACH TO DO THIS.

CIRM HAS INVESTIGATED -- HAS INVESTED IN OUR RESEARCH. WE HAVE AN EARLY TRANSLATION II GRANT WHERE WE NOW KNOW THAT WE CAN USE HUMAN EMBRYONIC STEM CELL-DERIVED POPULATIONS AS NEURAL STEM CELLS, TRANSPLANT THOSE INTO HD MICE, AND WE SEE A BENEFICIAL CLINICAL OUTCOME. WE HAVE A CLINICAL PATH FORWARD WITH THESE CELLS. THEY'RE GMP COMPATIBLE, AND WE SEE VERY STRONG EVIDENCE OF NEUROPROTECTION.

THIS APPLICATION PROVIDES A NEW STRATEGY AND ADDITIONAL BENEFIT IN THAT WE'RE GOING TO TAKE THOSE LINES, ENGINEER THEM TO PUMP OUT A THERAPEUTIC PROTEIN. SO THIS PROTEIN DIRECTLY TARGETS THE MUTANT HUNTINGTON PROTEIN, WHICH IS THE CAUSATIVE AGENT IN HD. IT DIRECTLY TARGETS IT. WE KNOW THAT WE CAN PUT THIS INTO MICE AND CHANGE PATHOLOGY IN THE BRAIN. AND SO IT GIVES ADDITIONAL BENEFIT. NOT ONLY CAN WE TREAT THE DISEASE WITH THE NEURAL STEM CELL TRANSPLANTATION, BUT WE CAN THEN PUMP THIS THERAPEUTIC PEPTIDE INTO THE BRAIN. IT HAS THE SURPRISING PROPERTY THAT IT'S TAKEN UP BY NEIGHBORING CELLS. IT GETS INTO CELLS AND CHANGES THE COURSE OF THE DISEASE IN THOSE CELLS. IT ALSO

PROTECTS THE TRANSPLANTED CELLS THEMSELVES.

ONE OF THE ISSUES IS THAT YOU MAY TAKE ON DISEASE PHENOTYPES FROM THE DISEASED TISSUE INTO THE TRANSPLANTED CELLS, AND THIS PROTECTS THOSE TRANSPLANTED CELLS.

NOW, WE'RE VERY CLOSE TO THE PAYLINE, AS I SAID. I DIDN'T SUBMIT DATA FOR RECONSIDERATION ALTHOUGH WE DO HAVE NEW DATA BECAUSE IT'S NOT YET PUBLISHED, IT'S BRAND-NEW DATA, BUT THIS APPROACH IS FEASIBLE. IT LEVERAGES CIRM'S INVESTMENT ALREADY IN THIS WORK BY ADDING ADDITIONAL BENEFIT TO THE PATIENTS. IT'S AN UNMET CLINICAL NEED, AND I THINK THIS WOULD BE A VERY IMPORTANT AND POTENTIALLY TRANSFORMATIVE AND INFORMATIVE APPROACH, NOT ONLY FOR HUNTINGTON'S DISEASE, BUT ALSO FOR ALZHEIMER'S DISEASE, PARKINSON'S DISEASE, AND OTHER NEURODEGENERATIVE DISEASES. AND I'M HERE REQUESTING THAT IF THERE'S FUNDING AVAILABLE, THAT THIS GET MOVED UP ONE. THANK YOU SO MUCH FOR YOUR TIME, AND I APPRECIATE IT.

MR. SHEEHY: THANK YOU. SO WE HAVE A MOTION ON THE FLOOR. IS THERE ANYONE ON THE BOARD WHO WOULD LIKE TO SPEAK TO THE MOTION? THEN NO OTHER PUBLIC COMMENT?

MS. SAMUELSON: I JUST HAD A QUESTION,

JEFF.

MR. SHEEHY: CAN WE COME BACK AFTER THE VOTE, JOAN, PLEASE? THANK YOU. MARIA, WOULD YOU LIKE TO --

DR. DULIEGE: MAYBE IN ADDITION TO THE ONE JUST MADE, CAN WE GET --

MR. SHEEHY: WE HAVE A MOTION. SO I WOULD RATHER VOTE THE MOTION. IF YOU WANT TO TALK ABOUT IT AFTER THE MOTION. BUT PART OF WHY WE'VE GONE THROUGH ALL OF THESE CHANGES IS TO TRY TO STREAMLINE THIS PROCESS SO THAT WE'RE NOT ALWAYS SUBSTITUTING OUR JUDGMENT FOR THAT OF THE REVIEW GROUP AND ALSO FOR OUR SCIENTIFIC STAFF WHO DID HAVE AN OPPORTUNITY TO REVIEW THIS GRANT.

AND AT THIS POINT WE HAVE TO MAKE -- OUR FUNDS ARE NOT UNLIMITED AND WE HAVE TO HAVE SOME BASIS, AND IT DOESN'T NECESSARILY -- IF WE MAKE IT ADVANTAGEOUS FOR EVERYONE WHO DIDN'T GET A GRANT TO COME HERE TO GET A HEARING, AND IF PEOPLE WILL READ THROUGH THEIR REVIEWS, IF SOMETHING POPS OUT, AND THIS IS REALLY SUPPOSED TO BE FOR PROGRAMMATIC CONSIDERATION, AND I DO KNOW THAT WE HAVE A HUNTINGTON'S GRANT THAT IS VERY HIGHLY SCORED IN THE DEVELOPMENT CANDIDATE FIELD. AND SO THAT ONE IS LIKELY TO GET APPROVED AND IS MUCH CLOSER TO

TRANSLATION INTO PATIENTS, WHICH IS SUPPOSED TO BE THE PROGRAMMATIC CONSIDERATIONS THAT WE TYPICALLY TAKE AT THIS POINT. SO IF WE CAN GO TO A ROLL CALL, IT WOULD BE --

DR. DULIEGE: THANK YOU, JEFF, FOR YOUR VERY DIPLOMATIC RESPONSE.

MR. HARRISON: JEFF, JUST ONE REMINDER FOR THOSE MEMBERS WHO ARE ELIGIBLE TO VOTE. TO THE EXTENT THAT YOU HAVE A CONFLICT IN ANY APPLICATION AMONG THE DCF APPLICATIONS, PLEASE VOTE YES OR NO EXCEPT FOR THOSE APPLICATIONS IN WHICH YOU HAVE A CONFLICT.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: MARCY FEIT. MICHAEL GOLDBERG. STEVE JUELSGAARD.

MR. JUELSGAARD: YES.

MS. BONNEVILLE: SHERRY LANSING. FRANCISCO PRIETO.

DR. PRIETO: YES, EXCEPT FOR THOSE WITH WHICH I HAVE A CONFLICT.

MS. BONNEVILLE: ROBERT QUINT.

DR. QUINT: YES.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: YES, EXCEPT FOR THOSE WITH

WHICH I HAVE A CONFLICT.

MS. BONNEVILLE: JOAN SAMUELSON.

MS. SAMUELSON: ABSTAIN.

MS. BONNEVILLE: JEFF SHEEHY.

MR. SHEEHY: YES, EXCEPT FOR THOSE WITH WHICH I HAVE A CONFLICT.

MS. BONNEVILLE: OS STEWARD. JONATHAN THOMAS.

CHAIRMAN THOMAS: YES.

MS. BONNEVILLE: ART TORRES.

MR. TORRES: AYE.

MS. BONNEVILLE: DIANE WINOKUR.

MS. WINOKUR: AYE.

MS. BONNEVILLE: MICHAEL GOLDBERG.

MR. HARRISON: SINCE EACH OF THE APPLICATIONS HAS A DIFFERENT QUORUM REQUIREMENT, I RECOMMEND THAT YOU PROCEED WITH OTHER BUSINESS WHILE WE VERIFY THAT THE MOTION PASSED AS TO ALL THE APPLICATIONS.

MR. SHEEHY: THANK YOU. JOAN, YOU HAD A QUESTION.

MS. SAMUELSON: I HAD THOUGHT THAT ONE OF THE PROCEDURAL MODIFICATIONS WOULD ENABLE, FOR EXAMPLE, DR. BRYANT TO PROVIDE THE COMMENT THAT SHE HAD. AND I'M WONDERING WHY -- I GUESS I

MISUNDERSTAND IT. I'D JUST LIKE TO HAVE THAT CLARIFIED.

MR. SHEEHY: MAYBE WE COULD TAKE THAT UP AFTER WE GET THROUGH WITH CONSIDERATION OF THESE APPLICATIONS. AND I MEAN HISTORICALLY WE HAVE NOT HAD INDIVIDUALS FROM INSTITUTIONS WHOSE GRANTS WERE UNDER CONSIDERATION BE ABLE TO MAKE COMMENTS ON THEM. OUR PROCEDURE DIDN'T CHANGE IN THAT WAY.

MS. SAMUELSON: I GUESS I THOUGHT IT WAS IN THE CONTEXT OF A SITUATION WHERE THEY CAN PARTICIPATE IN THE DISCUSSION, BUT THEN NOT BE INCLUDED IN THE VOTE.

MR. SHEEHY: WE'LL TALK ABOUT AFTER. SO COULD WE MOVE TO THE DEVELOPMENT CANDIDATES? AND I THINK BECAUSE THERE WAS AN INTEREST, COULD SOMEONE FIND OUT IF MICHAEL GOLDBERG -- GET HIM BACK ON BECAUSE THOSE GRANTS ARE UNDER CONSIDERATION.

SO, AGAIN, SAME PROCESS. THE FIRST QUESTION IS WHETHER ANYONE WOULD WANT TO MOVE ANYTHING -- MAKE A MOTION TO MOVE ANYTHING OUT OF TIER I. AND IF THERE IS NO MOTION TO MOVE ANYTHING OUT OF TIER I, ARE THERE ANY MOTIONS TO MOVE SOMETHING FROM, WHICH I THINK IS JUST ALL TIER III HERE, TO MOVE ANYTHING FROM TIER III INTO TIER I.

AND SO I DO THINK MICHAEL GOLDBERG DID

HAVE SOMETHING ONLINE. ARE THERE ANY OTHER APPLICATIONS INDIVIDUALS HAVE AN INTEREST IN BESIDES THE ONE THAT MICHAEL GOLDBERG FLAGGED? I WONDER, SHOULD WE TAKE A BREAK AND GIVE MICHAEL A FIVE-MINUTE BREAK. I DON'T WANT TO DENY HIM THE OPPORTUNITY FOR A HEARING.

MS. SAMUELSON: SOUNDS LIKE A GOOD IDEA.

MR. GOLDBERG: CAN YOU HEAR ME IN THE ROOM?

MR. SHEEHY: I THINK THE FIRST THING THAT MIGHT BE HELPFUL IS TO GET A VOTE ON THE PRIOR MOTION, WHICH WAS THE -- MAYBE JAMES CAN LEAD YOU THROUGH THAT.

MR. HARRISON: MICHAEL, THE MOTION WAS TO APPROVE DCF APPLICATIONS IN TIER I AND NOT TO FUND THE REMAINING DCF APPLICATIONS, AND WE ASK YOU TO VOTE YES OR NO EXCEPT FOR THOSE WITH WHICH YOU HAVE A CONFLICT.

MR. GOLDBERG: YES, EXCEPT FOR THOSE WITH WHICH I HAVE A CONFLICT.

MR. SHEEHY: THANK YOU. NOW, MICHAEL, WE'RE IN THE DC CATEGORY, AND I BELIEVE THAT THERE WAS A GRANT THAT YOU HAD AN INTEREST IN THAT YOU WOULD LIKE TO MAKE A MOTION TO MOVE INTO THE FUNDABLE CATEGORY?

MR. GOLDBERG: THAT'S CORRECT.

MR. SHEEHY: PLEASE PROCEED.

MR. GOLDBERG: THAT WOULD BE 06888, WHICH WAS A PROPOSAL TO ENGINEER RESTORATION OF FUNCTION AFTER SPINAL CORD INJURY.

MR. SHEEHY: SO DO WE HAVE A SECOND ON THAT MOTION?

MR. TORRES: I'LL SECOND.

MR. SHEEHY: SENATOR TORRES HAS SECONDED. SO PERHAPS THE BEST THING MIGHT BE TO GET SOME INFORMATION FROM STAFF ABOUT THE APPLICATION.

DR. CANET-AVILES: STAFF IS DR. CANET-AVILES, AKA ROSA TOO.

SO THIS IS AN APPLICATION FOR A DEVELOPMENT CANDIDATE, AND IT'S FOCUSED ON A COMBINATION PRODUCT CONSISTING OF PROGENITOR CELLS ON A SCAFFOLD THAT COULD BE IMPLANTED INTO THE SPINAL CORD TO LIMIT THE FUNCTIONAL DEFICITS AFTER SPINAL CORD INJURY.

THE PRODUCT, THE COMBINATION PRODUCT, IS INTENDED TO MODIFY THE INJURED ENVIRONMENT OF THE SPINAL CORD TO BOTH PROMOTE, REPAIR, AND PROVIDE PROCESSES THAT NEEDED REPAIR. THE APPLICATION PROPOSES TO COMBINE TWO NEURAL CELL POPULATIONS OF DERIVED HUMAN INDUCED PLURIPOTENT STEM CELLS, WHICH

WOULD BE PATIENT DERIVED, WITH A BIODEGRADABLE SCAFFOLD. AND EACH OF THE COMPONENTS IN THE COMBINATION PRODUCT MAY BE ENGINEERED TO SECRETE NEUROTROPHIC FACTORS.

THE MILESTONES INCLUDE AN ITERATIVE EXAMINATION OF THE FUNCTIONAL EFFICACY OF THESE HIPS-DERIVED NEURAL STEM CELLS IN COMBINATION WITH THE SCAFFOLD AT MULTIPLE TIME POINTS AFTER THE INJURY IN A RODENT MODEL OF SPINAL CORD INJURY. GENETICALLY ENGINEERING THE NEURAL STEM CELLS TO SECRETE THESE NEUROTROPHIC FACTORS AND REPEATING THE STUDIES IN THE RODENT MODEL, IMPREGNATING THE SCAFFOLD WITH RELEASABLE NEUROTROPHIC FACTORS AS WELL AND REPEATING THE STUDIES IN THE RODENT MODEL. ADDING A SECOND HUMAN INDUCED PLURIPOTENT STEM CELL-DERIVED NEURAL CELL TYPE TO THE COMBINATION PRODUCT AND REPEATING THE STUDIES IN THE RODENT MODEL AND MOVING THE BEST CANDIDATE COMBINATION PRODUCTS FORWARD INTO THE CLINICAL DEVELOPMENT MODEL OF SPINAL CORD INJURY.

AS YOU CAN SEE, IT'S AN ITERATIVE PROCESS OF TRYING DIFFERENT CANDIDATES THERE.

SO THE REVIEWERS FOUND THAT THERE WAS, IN THE TERMS OF OBJECTIVES AND MILESTONES, A LACK OF GO/NO-GO DECISION POINTS. AND MOSTLY THE TARGET

PRODUCT DESCRIBED MULTIPLE POSSIBLE DEVELOPMENT CANDIDATES.

ANOTHER ASPECT THAT THE REVIEWERS FOUND WAS NOT VERY POSITIVE, THAT THE MULTIFUNCTIONAL APPROACH THAT THE TEAM PROPOSES IS RATIONAL, BUT IT WAS HIGHLY UNREALISTIC AND VERY AMBITIOUS.

THEY LIKED THE RESEARCH PLAN. AND WHILE THE REVIEWERS ACKNOWLEDGE THAT THE STUDIES PROPOSED COULD ADD SOME SIGNIFICANT KNOWLEDGE TO THE SPINAL CORD INJURY FIELD, THE SCIENTIFIC COMPLEXITY OF THE PLANNED COMBINATION PRODUCT LIMITED THEIR ENTHUSIASM.

THE SCOPE OF THE PROPOSAL WAS VIEWED AS TOO BROAD TO BE ACCOMPLISHED WITHIN THE THREE-YEAR PERIOD. AND I THINK THOSE WERE THE MAIN CONCERNS IN THE REVIEW. THEY FOUND THE TEAM TO BE EXCELLENT WITH EXTENSIVE EXPERIENCE IN NEURAL STEM CELLS AND TRANSPLANTATION INTO SPINAL CORD INJURY MODELS AND BIOMATERIAL DEVELOPMENT. SO THIS IS IT. THANKS.

MR. SHEEHY: ARE THERE QUESTIONS FOR STAFF ABOUT THIS GRANT? MICHAEL, DO YOU HAVE --

MR. GOLDBERG: IT'S MY UNDERSTANDING NOW THAT THE UNDERLYING SCAFFOLD THAT WAS PROPOSED IN THE APPLICATION WAS, SUBSEQUENT TO THE APPLICATION AND THE REVIEW, APPROVED FOR USE BY THE FDA,

SUBSTANTIALLY DERISKING THAT DIMENSION OF THE RESEARCH PROGRAM. I GUESS MY QUESTION WOULD BE IS THAT A RELEVANT FACTOR FOR CONSIDERATION?

DR. OLSON: OKAY. I'D LIKE TO ADDRESS THAT. THIS IS PAT OLSON. THE PRODUCT -- I MEAN I HOPE YOU HEARD WHAT THE PROPOSED PRODUCT -- WELL, THEY DON'T KNOW WHAT THE CANDIDATE IS YET. AND THAT'S PROBABLY OKAY, BUT YOU HAVE TO REALIZE THE COMPLEXITY OF WHAT'S BEING TALKED ABOUT.

SO WITH REGARD TO THE SCAFFOLD, THE SCAFFOLD IS BEING SUBMITTED FOR APPROVAL IN ANOTHER CONTEXT. SO, YES, WHEREAS IT IS A GOOD THING TO HAVE A SCAFFOLD THAT HAS BEEN APPROVED IN ONE CONTEXT, ITS USE IN THIS CONTEXT IS A SEPARATE COMBINATION PRODUCT. SO IT'S AT LEAST -- IT'S GOOD TO KNOW THAT IT'S APPROVABLE, BUT WHAT WE'RE TALKING ABOUT HERE IS WE'RE TALKING ABOUT EACH OF TWO POPULATIONS OF NEURAL STEM CELL-DERIVED PROGENITORS WHICH MAY OR MAY NOT BE MODIFIED WITH A GROWTH FACTOR, SO GENETICALLY MODIFIED, TWO CELL POPULATIONS MAY OR MAY NOT BE GENETICALLY MODIFIED, PLUS A SCAFFOLD WHICH MAY OR MAY NOT HAVE THERAPEUTIC PROTEINS ADDED TO IT.

NOW, THAT IN OF ITSELF IS FINE. TESTING ALL THOSE COMBINATIONS TO GET TO -- I THINK THE MAIN

CRITICISM THAT DR. CANET-AVILES HIGHLIGHTED FOR YOU, AT LEAST I WOULD, IS WHAT THE REVIEWERS SAID ABOUT FEASIBILITY OF MAKING THE CHOICE AMONG ALL THOSE POPULATIONS WITHIN A REASONABLE TIME FRAME AND THEN DOING ALL THE THINGS THAT YOU NEED TO DO FOR A DEVELOPMENT CANDIDATE WITH THE SELECTED PRODUCT.

SO I THINK THOSE -- IT HELPS TO KNOW THAT THE FDA WILL APPROVE A SCAFFOLD, BUT I THINK THE ISSUE IS THE VARIOUS COMBINATORIALS -- THE VARIOUS COMBINATIONS THAT ARE PROPOSED AND WHAT YOU MIGHT END UP WITH. AND THAT WAS THE REVIEWER'S ISSUE, FEASIBILITY.

MR. SHEEHY: J.T.

CHAIRMAN THOMAS: DR. OLSON, IT'S MY UNDERSTANDING THAT THIS CONCLUSION BY THE GRANTS WORKING GROUP WAS SUBMITTED FORMALLY IN OUR NEW APPEAL PROCESS. COULD YOU JUST ADDRESS THAT, PLEASE?

DR. OLSON: YES, IT WAS. AND STAFF DID REVIEW. IF YOU WILL GIVE ME A MOMENT PLEASE, I NEED TO PULL UP THE APPROPRIATE -- I JUST WANT OUR CONCLUSION. I BELIEVE HE CLAIMED -- GIL, YOU MIGHT HAVE THAT. OKAY. THE APPLICANT CONSULTED WITH THE REVIEW OFFICE AND DID -- SO DID HAVE A DISCUSSION WITH DR. SAMBRANO AND DID CHOOSE TO SUBMIT AN APPEAL

REQUEST BASED ON A MATERIAL DISPUTE OF FACT AND A REQUEST FOR RECONSIDERATION BASED ON MATERIAL NEW INFORMATION. THE REQUEST FOR RECONSIDERATION IS DENIED AS THE INFORMATION PROVIDED DOES NOT ADDRESS THE PRIMARY CONCERNS OF REVIEWERS.

SO I BELIEVE, AS HAS ALREADY BEEN NOTED, THAT THE ONE POINT HIGHLIGHTED IN THE REQUEST FOR RECONSIDERATION WAS THE FACT THAT THE SCAFFOLD WAS, AT LEAST BEING, I THINK, THE SUBJECT OF OR MOVING THROUGH THE CLINICAL STUDIES AND FDA APPROVAL PROCESS, BUT AS WE STATED, IT DOES NOT ADDRESS THE PRIMARY CONCERNS OF REVIEWERS. APPEAL WAS DENIED AS NO OBJECTIVELY VERIFIABLE FACT AND THE REVIEW SUMMARY WAS IDENTIFIED AND ALL CLAIMS OF ERROR REPRESENTED A DIFFERENCE OF SCIENTIFIC OPINION OR JUDGMENT.

SO THE POINT ABOUT THE SCAFFOLD WAS NOT THE PRIMARY POINT.

MR. SHEEHY: MICHAEL, DO YOU WANT TO SEE THE SCORES ON THIS? IS THAT HELPFUL FOR YOU?

DR. OLSON: I'D BE HAPPY TO GIVE YOU THE MEAN, MEDIAN, AND RANGE. WOULD THAT BE HELPFUL?

MR. GOLDBERG: YES. THANK YOU. ALSO THANK YOU FOR YOUR RESPONSE, DR. OLSON.

MR. SHEEHY: I THINK THAT WOULD BE

HELPFUL, THE SCORES.

DR. OLSON: SO THE MEAN SCORE WAS A 53, THE MEDIAN SCORE WAS A 55, THE RANGE OF SCORES WAS 40 TO 60. SO EVEN THE HIGHEST SCORE DID NOT FALL IN A TIER II RANGE. AND THE STANDARD DEVIATION, GIVEN THAT RANGE, WAS SEVEN.

MR. GOLDBERG: THANK YOU.

MS. SAMUELSON: QUESTION FOR DR. OLSON. IF THIS RFA IS MOVING TOO FAST FOR A GRANT APPLICATION OF THIS COMPLEXITY, AND THAT'S BASICALLY WHAT I UNDERSTOOD YOU SAYING, AND IF I'M WRONG, LET ME KNOW, THEN WHAT OTHER RFA -- THIS ISN'T A RHETORICAL QUESTION. I'M JUST CURIOUS.

DR. OLSON: I WASN'T SUGGESTING THAT IT WASN'T SUITABLE FOR THIS RFA. I WAS SUGGESTING THAT GIVEN THE MANY COMBINATIONS THAT WERE PROPOSED FOR TESTING AND THE ACTIVITIES REQUIRED ONCE YOU HAVE SELECTED A CANDIDATE TO ACTUALLY SORT OF MEET THE CRITERIA FOR HAVING CHOSEN A DEVELOPMENT CANDIDATE, THE APPLICANT MIGHT HAVE BEEN BETTER OFF PURSUING A DCF AWARD. AND THEN ONCE HAVING GOTTEN FURTHER ALONG MOVED FORWARD.

SO I DO BELIEVE THAT THE EARLY TRANSLATIONAL RFA INITIATIVE WAS THE APPROPRIATE INITIATIVE FOR THIS. IT'S NOT -- THE REVIEWERS'

OPINION WAS THAT THE ACTIVITIES CONTEMPLATED UNDER THIS AWARD WERE, AS THEY NOTED, A HUGE AMOUNT OF WORK TO BE DONE AND QUESTIONED WHETHER THE APPLICANT COULD GET THERE.

MR. SHEEHY: THANK YOU, DR. OLSON. DO WE HAVE FURTHER BOARD COMMENTS? ANY ADDITIONAL COMMENTS FROM STAFF, BOARD? I'M ASSUMING YOU HAVE PUBLIC COMMENT.

MR. GOLDBERG: YES.

MR. REED: THIS IS DON REED. FIRST OFF, I THINK THERE'S A GAP IN THE CIRM PORTFOLIO. IF YOU VISIT THE WEB SITE ON SPINAL CORD INJURY AT THE CIRM WEB SITE, YOU WILL SEE THAT THERE'S TEN PROJECTS OF WHICH ONLY FIVE ARE ACTIVE. IT SAYS 59 MILLION IS ALLOCATED, BUT OF THAT 48 MILLION HAS BEEN CALLED BACK. SO THEY'VE GOT 11 MILLION. SO THE ROMAN REED ACT IS TINY COMPARED TO CIRM, AND YET WE'VE FUNDED $15 MILLION WORTH OF SPINAL CORD INJURY RESEARCH AND CIRM RIGHT NOW IS STUCK WITH 11 MILLION. I THINK THERE'S A DEFINITE GAP THERE.

SECONDLY, THE IDEA OF HIGHLY AND UNREALISTICALLY AMBITIOUS, THAT'S A DESCRIPTION OF EVERYTHING THAT'S ACTUALLY GOING TO WORK BECAUSE EVERY PROJECT THAT'S GOING TO BE PROPOSED FOR SPINAL CORD INJURY IS GOING TO BE MULTIFACETED. IT JUST

HAS TO BE THAT WAY.

ALSO, WHEN YOU ARE DIAGNOSED PARALYZED, THE ONLY REALISM IN YOUR LIFE IS THEY TELL YOU THAT THERE IS NO HOPE. BUT CIRM IS ABOUT MAKING HOPE REAL THROUGH ACCOMPLISHMENTS. WE DON'T HAVE TO ASK OURSELVES WHAT CAN BE DONE WITH A DOLLAR FIFTY, BUT WHAT ARE PROMISING POSSIBILITIES BROUGHT BY SERIOUS INDIVIDUALS.

THE PEOPLE INVOLVED ARE SERIOUS. THE SCAFFOLDING IS DONE BY ONE OF THE PIONEERS IN THIS FIELD. THE PI IS NOT ONLY HIGHLY RESPECTED IN SPINAL CORD INJURY, BUT ALSO STEM CELL RESEARCH. HE IS THE CHAIR OF THE FDA CELL, TISSUE, AND GENE THERAPY COMMITTEE. NO ONE KNOWS THE COMPLICATIONS AND THE REASON FOR THEM AND HOW TO SOLVE THEM BETTER THAN THIS MAN.

I REALLY THINK WE NEED TO EITHER RECONSIDER THIS OR FIND A DIFFERENT SPOT FOR IT BECAUSE I THINK THE SPINAL CORD INJURY PROJECT ITSELF IS WORTHY OF BEING FUNDED. IF THERE TO BE A DIFFERENT CATEGORY FOR IT, SO BE IT. BUT ALSO WE JUST NEED TO HAVE SOMETHING. WE'RE RUNNING OUT OF MONEY, AND SPINAL CORD INJURY IS A HUGELY INFLUENTIAL -- IT'S THE SYMBOL OF THAT WHICH CANNOT BE CURED. AND IF WE CAN CURE IT OR EVEN EASE IT,

ALLEVIATE THE SUFFERING OF SO MANY PEOPLE, THAT WILL BE A HUGE THING FOR PART 2. THANK YOU FOR HEARING ME OUT.

MR. SHEEHY: THANK YOU, DON. ARE THERE ADDITIONAL PUBLIC COMMENTS? WE HAVE ONE HERE IN SAN FRANCISCO -- SAN DIEGO.

DR. CRANE: MY NAME IS DR. ANDREW CRANE. EVAN CAN'T BE HERE, SO I'M SPEAKING ON HIS BEHALF. I'M A SCIENTIST IN HIS LAB. AND HE ORIGINALLY ASKED ME TO READ THIS LETTER THAT WAS ADDRESSING THE POTENTIAL FDA HURDLES FOR GETTING THIS MULTIMODAL THERAPY APPROVED AND FELT THAT THIS WAS NOT RECOMMENDED FOR FUNDING BASED PRIMARILY ON THAT.

SO MAYBE THERE WAS A MISUNDERSTANDING. HE ASKED ME TO READ THIS LETTER, AND THE GIST OF THIS IS THAT, YES, THE SCAFFOLD IS BIODEGRADABLE AND IS FDA APPROVED AND ENTERING CLINICAL TRIALS FOR SPINAL CORD INJURY PATIENTS THROUGH A COMMERCIAL PARTNER, IN VIVO THERAPEUTICS. AND THIS COMPANY WOULD ALSO BE HELPING WITH OUR IND-ENABLING STUDIES. I GUESS THIS WAS NOT KNOWN TO THE GRANTS WORKING GROUP AT THAT TIME.

SIMILARLY, WE HAVE NEW DATA SHOWING, ACCORDING TO EVAN, THAT STEM CELLS ALONE DO NOT MAKE A SIGNIFICANT IMPACT ON SPINAL CORD INJURY. THE

SCAFFOLD ALONE DOES MAKE AN IMPACT, BUT IT'S NOT NEARLY AS IDEAL AS WHAT POTENTIALLY COULD BE WHEN THE THERAPIES ARE COMBINED. THERE IS A SYNERGY WITH STEM CELLS AND THE SCAFFOLD THAT ARE INCREASING THE BEHAVIORAL AND FUNCTIONAL RESPONSES IN THE SPINAL CORD INJURY MODEL.

SO WITH REGARD TO THE DATA ON THESE APPROACHES, WE PROVIDE THIS DATA SHOWING THAT THE COMBINATION IS AN IDEAL AVENUE FOR EXPLORATION. SO EVAN WOULD SIMPLY LIKE TO REQUEST THAT THE APPLICATION BE REASSESSED BY AN EXTERNAL SCIENTIFIC STUDY SECTION WITH THIS NEW INFORMATION AND CORRECT THE DATA IN EVIDENCE. THANK YOU.

MR. SHEEHY: SO THE APPLICANT IS REQUESTING RE-REVIEW. I THINK --

DR. SAMBRANO: THIS WAS PART OF WHAT WAS REVIEWED DURING THE APPEALS PROCESS. SO THESE POINTS HAVE BEEN ALREADY CAREFULLY CONSIDERED. AND THE CONCLUSION WE REACHED WAS THAT THE APPEAL DID NOT MEET EITHER THE CRITERIA OR DID NOT ADDRESS THE MAIN CONCERNS OF REVIEWERS. SO I DON'T THINK WHAT WE'VE HEARD CHANGES THAT.

MR. SHEEHY: IS THERE ANY OTHER ADDITIONAL PUBLIC COMMENT? SHOULD WE PROCEED TO A VOTE? AND THE VOTE IS TO APPROVE THIS APPLICATION FOR FUNDING.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.
DR. DULIEGE: YES.
MS. BONNEVILLE: MARCY FEIT. MICHAEL GOLDBERG.
MR. GOLDBERG: YES.
MS. BONNEVILLE: STEVE JUELSGAARD.
MR. JUELSGAARD: NO.
MS. BONNEVILLE: FRANCISCO PRIETO.
DR. PRIETO: NO.
MS. BONNEVILLE: ROBERT QUINT.
DR. QUINT: NO.
MS. BONNEVILLE: AL ROWLETT.
MR. ROWLETT: NO.
MS. BONNEVILLE: JOAN SAMUELSON.
MS. SAMUELSON: ABSTAIN.
MS. BONNEVILLE: JEFF SHEEHY.
MR. SHEEHY: NO.
MS. BONNEVILLE: OS STEWARD. JONATHAN THOMAS.
CHAIRMAN THOMAS: NO.
MS. BONNEVILLE: ART TORRES. DIANE WINOKUR.
MR. HARRISON: WHILE WE'RE WAITING FOR MEMBERS WINOKUR AND TORRES TO RETURN TO --
MR. TORRES: AYE.

MR. SHEEHY: SENATOR TORRES VOTES AYE.

MR. HARRISON: THE MOTION FAILS BY A VOTE OF THREE YES VOTES TO SIX NO VOTES.

AND JUST TO CONFIRM, THE PRIOR MOTION ON THE DCF APPLICATIONS WAS VALID, ALL THE APPLICATIONS.

MR. SHEEHY: ARE THERE ANY ADDITIONAL MOTIONS TO MOVE AN APPLICATION FROM TIER III INTO TIER I? ARE WE READY FOR A GLOBAL MOTION MUCH LIKE THE LAST ONE? COULD YOU MAYBE GIVE US THE FORM?

MR. HARRISON: YES. THE MOTION WOULD BE TO APPROVE THE DC APPLICATIONS IN TIER I AND NOT TO FUND THE REMAINING APPLICATIONS.

MR. SHEEHY: SO COULD I GET A MAKER AND A SECOND?

DR. DULIEGE: SO MOVED.

MR. JUELSGAARD: SECOND.

MR. SHEEHY: THE SECOND IS STEVE JUELSGAARD. AND THEN THE SAME FORM AS BEFORE. ANNOUNCE YOUR CONFLICTS, SO TO SPEAK. ANY PUBLIC COMMENT ON ANY REMAINING APPLICATIONS IN THIS CATEGORY EITHER HERE OR IN PALO ALTO? NO PUBLIC COMMENT. THEN WE'LL GO TO A ROLL CALL.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: YES.

MS. BONNEVILLE: MARCY FEIT. MICHAEL GOLDBERG.

MR. GOLDBERG: YES, EXCEPT FOR THOSE WITH WHICH I HAVE A CONFLICT.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: YES.

MS. BONNEVILLE: SHERRY LANSING. FRANCISCO PRIETO.

DR. PRIETO: YES, EXCEPT FOR THOSE WITH WHICH I HAVE A CONFLICT.

MS. BONNEVILLE: ROBERT QUINT.

DR. QUINT: YES.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: YES, EXCEPT FOR THOSE WITH WHICH I HAVE A CONFLICT.

MS. BONNEVILLE: JOAN SAMUELSON.

MS. SAMUELSON: ABSTAIN.

MS. BONNEVILLE: JEFF SHEEHY.

MR. SHEEHY: YES, EXCEPT FOR THOSE WITH WHICH I HAVE A CONFLICT.

MS. BONNEVILLE: OS STEWARD. JONATHAN THOMAS.

CHAIRMAN THOMAS: YES.

MS. BONNEVILLE: ART TORRES.

MR. TORRES: AYE.

MS. BONNEVILLE: DIANE WINOKUR.

MR. HARRISON: IF STAFF COULD TRY TO FIND MEMBER WINOKUR SO WE COULD RECORD HER VOTE ON THIS MOTION, THAT WOULD BE GREAT.

DR. FINE: AM I ON YOUR LIST?

MR. HARRISON: NO. WE ONLY CALL MEMBERS OF THE APPLICATION REVIEW SUBCOMMITTEE WHO ARE ELIGIBLE TO VOTE. AND AS AN INSTITUTIONAL MEMBER YOU ARE NOT.

CHAIR, I'D SUGGEST THAT WE LEAVE THE ROLL CALL OPEN AS WE'RE PERMITTED TO DO UNDER THE BOARD'S BYLAWS UNTIL AFTER THE LUNCH BREAK, AND THEN WE CAN THEN HAVE MEMBER WINOKUR RECORD HER VOTE AT THAT TIME IF THAT'S ACCEPTABLE TO THE BOARD.

MR. SHEEHY: J.T., I THINK THIS IS YOUR CALL, NOT MY CALL.

CHAIRMAN THOMAS: THAT IS ACCEPTABLE. FOR MEMBERS OF THE PUBLIC, WE ARE GOING TO BREAK FOR LUNCH AND CLOSED SESSION AT THE MOMENT. I THINK YOU HAVE A SENSE OF WHERE THE VOTE IS, SO I DON'T THINK IT'S NECESSARY FOR YOU TO STICK AROUND UNTIL CLOSED SESSION IS OVER BECAUSE THAT WILL BE A LITTLE WHILE.

SO I WOULD LIKE TO ECHO THE COMMENTS OF MR. SHEEHY. THANK YOU, ALL OF YOU, FOR COMING FOR YOUR COMMENTS, FOR SHARING YOUR STORIES. WE GREATLY

APPRECIATE IT.

AND SO BEFORE MARIA SPEAKS ON THE ALL IMPORTANT ISSUE OF WHERE LUNCH IS, JAMES, IF YOU COULD JUST ADMONISH US AS TO THE BASIS FOR THE CLOSED SESSION.

MR. HARRISON: THE BOARD WILL BE CONVENING IN CLOSED SESSION TO DISCUSS PERSONNEL PURSUANT TO HEALTH AND SAFETY CODE SECTION 125290.30(F)(3)(D).

CHAIRMAN THOMAS: MARIA.

MS. BONNEVILLE: FOR BOARD MEMBERS LUNCH IS IN THE PACIFIC ROOM, WHICH IS THAT WAY. GO OUT AND TURN LEFT AROUND THE CORNER TOWARDS THE END. AND STAFF, WE WILL HAVE LUNCH WHERE WE HAD BREAKFAST THIS MORNING.

DR. FINE: WHAT TIME DO YOU WANT US TO LOG IN FOR PHONE COMMUNICATION? THERE'S A SEPARATE CONNECTION REQUIRED. WHAT TIME DO YOU WANT US TO OPEN THAT CONNECTION?

CHAIRMAN THOMAS: I WOULD SAY FIVE MINUTES.

DR. FINE: OKAY. THANK YOU.

CHAIRMAN THOMAS: THANK YOU, THOSE ON THE PHONE. WE WILL NOW ADJOURN TO CLOSED SESSION AND SEE EVERYBODY AT ITS CONCLUSION.

(THE APPLICATION REVIEW SUBCOMMITTEE

WAS THEN ADJOURNED, AND THE FULL BOARD THEN CONVENED IN CLOSED SESSION, NOT REPORTED NOR HEREIN TRANSCRIBED.)

CHAIRMAN THOMAS: EVERYBODY PLEASE TAKE YOUR SEATS. MEETING IS NOW CALLED BACK TO ORDER. I THINK THE FIRST ORDER OF BUSINESS IS, DIANE, WE NEED -- JAMES, WHICH VOTE DO WE NEED DIANE'S FINAL TALLY ON?

MR. HARRISON: WE NEED DIANE'S VOTE ON THE MOTION TO APPROVE THE DC APPLICATIONS IN TIER I AND NOT TO FUND THE REMAINING APPLICATIONS.

MS. WINOKUR: MY VOTE IS YES.

CHAIRMAN THOMAS: DIANE VOTES YES.

SO, MR. HARRISON, THE RESULTS OF THE VOTE ARE?

MR. HARRISON: THE MOTION CARRIES.

CHAIRMAN THOMAS: THANK YOU. THAT CONCLUDES THE EARLY TRANSLATION IV DISCUSSION. I WOULD LIKE TO PUBLICLY THANK MR. SHEEHY FOR HIS GREAT LEADERSHIP. THIS IS THE FIRST MAJOR DISCUSSION THAT WE'VE HAD UNDER THE NEW PROTOCOL WITH PROGRAMMATIC REVIEW BEING AT THE BOARD, AND I THOUGHT IT WENT EXTREMELY WELL. JEFF, THANK YOU FOR YOUR WORK IN GUIDING US THROUGH. LET THE RECORD SHOW WE THANKED MR. SHEEHY.

MR. SHEEHY: THANK YOU TO THE CHAIR.

CHAIRMAN THOMAS: NEXT WE'RE PROCEEDING TO ACTION ITEM NO. 11, CONSIDERATION OF PROPOSED PROGRAM ANNOUNCEMENTS FOR CIRM INDUSTRY CO-FUNDING AGREEMENT. ELONA HAS THE PODIUM.

MS. BAUM: THANK YOU VERY MUCH FOR YOUR ATTENTION. AND WHAT I WOULD LIKE TO DO IS BRIEFLY DESCRIBE A CONCEPT PROPOSAL FOR A PROGRAM ANNOUNCEMENT WHICH WOULD SEEK THE CREATION OF INDUSTRY COLLABORATION AND CO-FUNDING AGREEMENTS WITH CIRM AND ELIGIBLE INDUSTRY REPRESENTATIVES.

SO BEFORE I START WITH THE PURPOSE, I JUST WANTED TO GIVE A LITTLE BIT OF BACKGROUND ABOUT OUR STRATEGIC PARTNERSHIP FUNDING RFA'S.

(INTERRUPTION IN PROCEEDINGS.)

CHAIRMAN THOMAS: HOLD ON ONE SECOND. TECHNOLOGY GURU AMY IS ON HER WAY OVER TO FIGURE OUT WHAT'S WRONG.

MS. BAUM: OKAY. JUST TO SET THE STAGE AND PROVIDE A LITTLE BIT OF BACKGROUND, OUR STRATEGIC PARTNERSHIP FUNDING RFA'S HAVE A COMMERCIALIZATION VALIDATION REQUIREMENT AS AN ELIGIBILITY REQUIREMENT. AND WHAT THAT PROVIDES IS THAT IN ORDER TO BE ELIGIBLE FOR ENTRY INTO THAT RFA, YOU EITHER HAVE TO HAVE AN AGREEMENT WITH AN

INDUSTRY COLLABORATOR, AND WE'VE DESCRIBED THAT COLLABORATOR AS HAVING TO HAVE A MARKET CAP OF 500 MILLION, OR YOU HAVE TO HAVE RAISED, AND IT'S CHANGED BETWEEN RFA'S, EITHER 10 MILLION IN THE PAST TWO YEARS OR 15 MILLION IN THE PAST TWO YEARS. IF YOU CAN SATISFY THAT REQUIREMENT, WE SAY YOU'VE ESTABLISHED COMMERCIAL VALIDATION AND YOU AT LEAST CAN BE ELIGIBLE VIS-A-VIS THOSE REQUIREMENTS.

AND SO WHAT THE PURPOSE OF THIS PROPOSED AGREEMENT VIA THE PROGRAM ANNOUNCEMENT IS IS TO MAKE IT WIDELY KNOWN TO THE INDUSTRY THAT WE ARE INTERESTED IN ENTERING INTO THESE AGREEMENTS WITH ALL QUALIFIED ENTITIES; I.E., PHARMA THAT HAS A 500 MILLION MARKET CAP OR BIOPHARMACEUTICAL THAT HAS A 500 MARKET CAP AND VENTURE FIRMS WITH THE QUALIFYING CRITERIA TO BE DETERMINED AT A LATER DATE IN THE RFA OR THE PROGRAM ANNOUNCEMENT.

AND I THINK THE REASON IS HOPEFULLY SELF-EVIDENT. IT'S, IN ESSENCE, SO WE CAN JUMP-START THE PROCESS BECAUSE WHAT WE HAVE SEEN IN PRACTICE IS THAT WE POST THE RFA AND THEN THE DISCUSSIONS BETWEEN THE CALIFORNIA RESEARCHERS AND THE INDUSTRY PARTNERS START BEGINNING JUST THEN. WELL, WE ALL KNOW IT TAKES A LOT LONGER TIME THAN FOUR MONTHS, WHICH IS THE TYPICAL OR SIX MONTHS

WHICH IS A TYPICAL SPAN FROM POSTING THE RFA TO COMING TO THE ICO TO ACTUALLY CONCLUDE THESE NEGOTIATIONS. SO I FEEL LIKE WE'RE OFTEN BEHIND THE EIGHT BALL IN TERMS OF KNOWING WHETHER OR NOT THESE AGREEMENTS EVER WILL COME INTO FRUITION.

SO AS I SAID, WHAT WE'RE TRYING TO DO WITH THESE AGREEMENTS NOW IS TO JUMP-START THE PROCESS AND LET, IF THERE ARE ANY INDUSTRY COLLABORATORS OUT THERE WILLING TO ENGAGE WITH US, LET THEM KNOW THAT WE'D LIKE TO ENTER INTO THE PROCESS EARLY. AND LET ME PROVIDE YOU WITH SOME PROVISIONS AS TO WHAT THESE AGREEMENTS WOULD LOOK LIKE. BUT BEFORE I DO THAT ACTUALLY, I ALSO WANT TO TELL YOU WHAT THEY WON'T LOOK LIKE.

THIS IS NOT A CO-FUNDED RFA. SO WHAT THAT WILL MEAN IS THAT CIRM WILL CONTROL THE PROCESS IN EVERY SHAPE AND FORM. AND IT'S ALSO NOT INTENDED TO BE IDENTICAL TO WHAT WE HAVE RIGHT NOW WITH GOVERNMENT ENTITIES, THE COLLABORATIVE FUNDING PARTNER PROGRAM. BUT WHAT IT WILL DO IS THE FOLLOWING. SO AS I SAID, IT WILL BE OPEN TO QUALIFIED BIOPHARMAS, VC'S. AND WHAT IT WOULD PROVIDE FOR IS THAT EACH ENTITY, CIRM AND THE PARTNER, WOULD ONLY FUND PROGRAMS OF MUTUAL INTEREST. SO IT DOESN'T REQUIRE THE PHARMA TO FUND

SOMETHING THEY'RE NOT INTERESTED IN; IT DOESN'T REQUIRE CIRM TO FUND SOMETHING THAT THEY'RE NOT INTERESTED IN.

IN TERMS OF THE RIGHTS OR BENEFITS THE PARTNER WOULD RECEIVE, THEY WOULD HAVE INPUT INTO THE STRATEGIC PARTNERSHIP CONCEPTS THAT WE POST AND PRESENT TO THE BOARD; BUT, OF COURSE, THE INPUT DOES NOT HAVE TO BE ACCEPTED BY CIRM. AND WHAT IT WOULD MEAN IS THAT IF THIS IS ACCEPTED, WE WOULD KNOW WHO OUR INTERESTED PARTNERS ARE. WE'D BE ABLE TO IDENTIFY THEM ON OUR WEB SITES, AND THEN WE COULD TAKE PROACTIVE STEPS TO MATCH OUR RESEARCHERS WITH THESE ORGANIZATIONS THAT ARE INTERESTED IN DOING SOME CO-FUNDING WITH US. WE MIGHT EVEN ENGAGE IN WORKSHOPS WITH THEM OR OTHER EVENTS, BUT NOTHING SET IN STONE AT THIS TIME.

I ALSO WANT TO EMPHASIZE THAT WE CAN UNDER THIS PROGRAM ANNOUNCEMENT HAVE MULTIPLE INDUSTRY AGREEMENTS. IT DOESN'T HAVE TO BE WITH JUST ONE COMPANY AND ONLY ONE COMPANY. AND ALSO I WANT TO EMPHASIZE THAT THE RESEARCHERS IN CALIFORNIA CAN COME IN WITH A DESIGNATED INDUSTRY CO-FUNDING PARTNER OR THEY CAN COME UP WITH ANYBODY ELSE AS LONG AS THEY CAN SATISFY THE COMMERCIALIZATION VALIDATION REQUIREMENT. SO THIS REALLY JUST IS

TRYING TO ENHANCE THE STRATEGIC PARTNERSHIP PROGRAM, BUT THERE ARE MULTIPLE WAYS IN WHICH CALIFORNIA RESEARCHERS CAN COME IN.

AND ALSO IT BEARS TO BE EMPHASIZED THAT OUR FUNDING STRUCTURE REMAINS THE SAME. IT'S THE SAME GWG REVIEW, IT'S THE SAME BOARD APPROVAL. NOTHING CHANGES IN THAT REGARD.

I ALSO WANT TO EMPHASIZE THAT IN TERMS OF THE INDUSTRY PARTNER'S ACCESS TO CONFIDENTIAL INFORMATION, THIS IN MANY WAYS IS DIFFERENT THAN OUR CFP PROGRAM BECAUSE HERE CIRM DOESN'T GIVE ACCESS TO OTHER POTENTIAL PROGRAMS TO AN INDUSTRY PARTNER. SO THEY ARE NOT SITTING IN OUR GRANTS WORKING GROUP REVIEWS. WE NEVER GET IN THE MIDDLE OF PROVIDING ANY CONFIDENTIAL INFORMATION. IT IS STRICTLY UP TO THE CALIFORNIA RESEARCHER TO PROVIDE ACCESS TO THE INFORMATION IF THEY SO DESIRE AS REQUESTED BY THE INDUSTRY COLLABORATOR, AND THE COLLABORATOR DOES THEIR OWN DUE DILIGENCE.

AND I ALSO WANTED TO EMPHASIZE THAT ALL IP REGULATIONS WILL BE IN PLACE AND WILL APPLY AS WELL.

SO I THINK THERE MIGHT BE SOME QUESTIONS AS TO HOW THIS WILL WORK IN PRACTICE, SO I ACTUALLY INCLUDE IN MY SET OF SLIDES A FLOWCHART, AND I'M HAPPY TO GO THROUGH THE FLOWCHART IF THAT IS OF

INTEREST.

HEARING NO QUESTIONS, I THINK I'LL DO THAT. THE WAY IT WOULD WORK, AND THIS IS JUST ONE EXAMPLE, IS THAT, OKAY, WE'RE SUCCESSFUL. YOU ALL VOTE AND SUPPORT THIS. WE ACTUALLY ENTER INTO AN AGREEMENT WITH A BIOPHARMA. THEN WHAT CIRM DOES IS FIND WAYS TO CREATE LINKAGES WITH THE BIOPHARMA OR THE VC AND CALIFORNIA ENTITY. SO WE DO THAT BY POSTING ON OUR WEB SITE. GEE, COMPANIES A, B, AND C ARE INTERESTED IN THERAPEUTIC AREAS X, Y, AND Z. AND HOPEFULLY THAT HELPS FACILITATE SOME OF THE LINKAGES. AND, AGAIN, WE CAN SURMISE OTHER INPUTS.

AT SOME POINT IN TIME, WE ALSO ASK THESE ENTITIES IF THEY HAVE ANY INPUT THEY WANT TO PROVIDE TO THE NEXT ROUND OF STRATEGIC PARTNERSHIP RFA'S. AS YOU KNOW, THE CONCEPT PLANS ARE VERY, VERY HIGH LEVEL. SO I WOULDN'T SURMISE THAT THEY WOULD HAVE A LOT OF PARTICULAR INPUT, BUT THEY MIGHT WANT TO INDICATE AT LEAST WHAT AREAS THAT THEY'RE INTERESTED IN, AND WE DON'T HAVE TO LISTEN TO THAT. THEN, OF COURSE, AS I INDICATED, HOPEFULLY THROUGH OUR LINKAGES EFFORTS, THESE PARTNERSHIPS ARE CREATED, THERE'S SOME INTEREST CREATED BETWEEN CALIFORNIANS AND THE FUNDING PARTNERS, AND THE FUNDING PARTNER STARTS DOING ITS DUE DILIGENCE AND NEGOTIATES A

POTENTIAL DEAL WITH THEM.

SO WHILE THE NEGOTIATIONS ARE ONGOING WITH THE POTENTIAL CALIFORNIA RESEARCHER, CIRM POSTS ITS RFA. AND THEN THE CALIFORNIA COMPANY COULD COME IN WITH A LETTER OF INTENT THAT'S SIGNED BY THIS POTENTIAL COLLABORATOR, WHICH IS WHAT WE'VE ALWAYS REQUIRED IS LETTER OF INTENT AT THE ENTRY STAGE, AND THEY WILL BE CONTINUING TO NEGOTIATE WHILE THE GWG ACTUALLY EVALUATES THE PROJECT. AND MEANWHILE THE COLLABORATOR IS EVALUATING THEIR DUE DILIGENCE. AND THEN IT COULD BE THAT, IF ALL GOES WELL, THE INDUSTRY PARTNER AND THE CALIFORNIA RESEARCHER ENTER INTO AN AGREEMENT AND ULTIMATELY, ONCE THAT AGREEMENT IS ENTERED INTO AND IF THE PROJECT HAS RECEIVED AN ELIGIBLE SCORE, IT WILL GO TO THE ICOC, BUT WE WOULD NOT BRING TO THE ICOC THOSE PROJECTS UNLESS AND UNTIL THE AGREEMENT HAS BEEN ENTERED INTO BETWEEN THE CALIFORNIA RESEARCHER AND THE FUNDING ENTITY.

SO THAT'S IN CONCLUSION THE WAY THAT THIS PROCESS IS ENVISIONED, AND IT'S MY HOPE THAT THIS BOARD WOULD SUPPORT THIS AND LET US POST THIS PROGRAM ANNOUNCEMENT AS DEFINED.

DR. WESTON: WHAT WAS YOUR THINKING ABOUT PUTTING A CAPITALIZATION LIMIT AT 500 MILLION? AND

WHO DO YOU THINK THAT'S GOING TO EXCLUDE BY DOING THAT?

MS. BAUM: WE DID SOME ANALYSIS IN THE VERY BEGINNING BECAUSE WE WERE GETTING SOME CALIFORNIA RESEARCHERS THAT WERE SAYING THAT ENTITIES HAVING $100 MILLION MARKET CAP WAS THEIR PARTNER. AND THE THOUGHT IS WE WANTED COMPANIES THAT SEEMED THAT THEY HAD AT LEAST A SUFFICIENT LEVEL OF RESOURCES TO BE ABLE TO QUALIFY. AND LONG AGO WE DECIDED ON THE 500 BECAUSE THE WHOLE POINT OF IT IS IDEALLY TO GET SOME LEVERAGE FOR THE CURRENT PROJECT, BUT IDEALLY ALSO TO HAVE STRONG BACKING SO THAT THE PARTNER COULD HOPEFULLY PROVIDE SOME FOLLOW-ON FUNDING ONCE THE CIRM-FUNDED PROJECT IS COMPLETED.

MR. SHEEHY: WHO CONTROLS THE FATE OF THESE PROJECTS? I WOULD BE CONCERNED THAT WE WOULD BE PUTTING MONEY INTO A PROJECT, THE COMPANY WOULD BE PUTTING MONEY INTO THE PROJECT, BUT THE ULTIMATE CONTROL OVER WHAT HAPPENS TO THESE PROJECTS WOULD THAT NOT LIE WITH THE COMPANY? AND SO IF THEY DECIDED THAT FOR WHATEVER STRATEGIC REASON THEY HAD THAT THEY WEREN'T INTERESTED IN INVESTING ANYMORE, HAVING MADE THAT INVESTMENT, I'M NOT COMPLETELY SURE THEY WOULD NECESSARILY BE INTERESTED JUST GIVING IT

TO THE WORLD. I JUST WONDER. WE PUT 10 MILLION INTO A PROJECT, THEY PUT 10 MILLION INTO A PROJECT OR THEY PUT FIVE AND FIVE. I THINK IT WAS ABOUT 10 MILLION IS WHAT WE'RE DOING FOR THE STRATEGIC PARTNERSHIPS.

PRODUCT DEVELOPMENT IS REPLETE WITH SITUATIONS WHERE PEOPLE HAVE DEVELOPED PRODUCTS WITHIN COMPANIES, BUT THEY DIDN'T MEET THE LONG-TERM STRATEGIC VISION, AND THEY JUST PUT THEM ON THE SHELF AND THEY SAT ON THE SHELF. HOW DO WE ADDRESS THAT PROBLEM?

MS. BAUM: SO THAT'S WHY I MENTIONED OUR IP REGULATIONS BECAUSE WE DO HAVE MARCH-IN RIGHTS UNDER OUR IP REGULATIONS THAT APPLY, AND THOSE WOULD BE REFERENCED IN THE ACTUAL AGREEMENT WITH THE INDUSTRY COLLABORATORS.

MR. SHEEHY: I DON'T THINK THAT THAT'S -- I MEAN, WITH ALL DUE RESPECT, I DON'T THINK THAT'S WHAT THOSE MARCH-IN RIGHTS WERE DESIGNED FOR. AND I THINK THAT WAS A VERY CONTROVERSIAL ELEMENT IN OUR IP REGULATIONS BACK WHEN WE MADE THEM. I HATE TO KEEP SAYING DUANE ROTH, BUT AROUND THE CORNER HERE IN DUANE ROTH'S OFFICE WAY BACK IN THE DAY WHEN ED PENHOET WAS CHAIRING THOSE MEETINGS. BUT ONE OF THE MOST SENSITIVE AREAS THAT WE WERE DISCUSSING WERE

MARCH-IN RIGHTS. AND OUR COMMUNICATION AT THE TIME WAS THAT WE WOULD BE VERY RELUCTANT TO USE THOSE UNLESS THERE WAS AN ABSOLUTE NECESSITY, MUCH LIKE BAYH-DOLE HAS ONE FOR THE FEDERAL GOVERNMENT, PUBLIC HEALTH. WE OBVIOUSLY DIDN'T WANT THINGS TO MOLDER.

BUT I WOULD BE CONCERNED HERE THAT WE WOULD BE SETTING UP A SITUATION WHERE OUR ONLY RECOURSE TO MAKING SURE THE PRODUCTS ARE DEVELOPED THAT WE HAD INVESTED IN IS TO INVOKE OUR MARCH-IN RIGHTS WHICH I THINK WOULD TERRIFY ANY OTHER COMPANY FROM ENGAGING WITH US IN ANY OTHER SCENARIO BECAUSE THEY'D SEE US AS BASICALLY WALKING AROUND WITH A LOADED GUN ALL THE TIME.

MS. BAUM: THE AGREEMENT ITSELF WOULD HAVE PROVISIONS THAT TALK ABOUT WHAT TO DO IN THE COURSE OF MIDWAY THROUGH A MILESTONE ISN'T MET AND WHO CAN TERMINATE AND UNDER WHAT CONDITIONS AND WHO WOULD END UP HAVING ACCESS AND THE ABILITY TO CONTINUE DEVELOPMENT OF IT. SO THESE AGREEMENTS WOULD HAVE PROVISIONS SUCH AS THAT.

AND THEN WHAT YOU SAY IS NO DIFFERENT THAN WHAT WOULD HAPPEN NO MATTER WHAT WITH ANY INDUSTRY PARTNER. IF THEY OWN THE IP AND THEY RECEIVE OUR FUNDING, THEY'RE OBLIGATED TO FIND SOME FORM OF PRACTICAL APPLICATION UNDER OUR REGULATIONS. BUT

WHAT YOU'RE SAYING MEANS THAT WE WOULD NEVER EVER FUND ANY COMPANY BECAUSE SOMETIMES COMPANIES OWN IP.

MR. SHEEHY: I JUST WOULD LIKE TO BE A LITTLE MORE CLEAR WHAT WE'RE TALKING ABOUT BECAUSE WE'RE GOING FROM A CASE-BY-CASE BASIS TO ACTUALLY GETTING INTO FORMAL PARTNERSHIPS WITH COMPANIES. ARE THERE SPECIFIC COMPANIES WHO ARE INTERESTED IN THIS?

MS. BAUM: I THINK ALAN HAS A COMMENT.

CHAIRMAN THOMAS: LET DR. TROUNSON ANSWER IT, THEN ELONA, THEN MR. JUELSGAARD.

DR. TROUNSON: SO THE WAY I ENVISAGE IT IS THAT IT NEEDS TO BE A JOINT FUNDING ARRANGEMENT. SO I'VE BEEN LOOKING AT THIS AS AN OPPORTUNITY TO LEVERAGE OUR FUNDING TO HELP GO THE FURTHER DISTANCE. SO I THINK IN A JOINT FUNDING SITUATION, WE WOULD HAVE THE SAME KIND OF CONTRACTUAL ARRANGEMENTS WORKING WITH ANY KIND OF COMPANY IN SUPPORT OF THAT PROJECT.

I THINK WHEN OUR FUNDING IS FINISHED, AND THE COMPANY WANTS TO TAKE IT ON, YEAH, WE REALLY PROBABLY DON'T HAVE A PLACE FOR THAT. BUT THAT'S GOING TO BE THE CASE, I GUESS, MOST OF THE TIME WHEN WE GET TO PHASE II.

SO I HAVE BEEN ENVISAGING THIS AS A SORT

OF JOINT FUNDING ARRANGEMENT. THAT'S THE WAY I'VE SEEN IT, THAT THERE ARE SOME COMPANIES WHO HAVE AN INTEREST IN COMING INTO SOME OF THE PROJECTS, BRING AN INTEREST INTO THE PROJECTS ON A BASIS THAT THEY WOULD GO THE DISTANCE OF THAT GRANT, BUT WHO KNOWS WHAT'S GOING TO HAPPEN NEXT. DEPENDS ON WHETHER THAT PROJECT WAS REALLY WORKING WELL OR NOT. AND THAT'S PRETTY MUCH MOST OF OUR ARRANGEMENTS ARE ON THAT BASIS.

I ACTUALLY DON'T SEE US JUST GIVING OVER THE PROJECT TO THAT COMPANY ALONE. I DON'T SEE THAT AS THE APPROPRIATE VEHICLE IN MY OWN VIEW, BUT I THINK THE BOARD WOULD NEED TO DECIDE. BUT I SEE IT AS A JOINT FUNDING ARRANGEMENT FOR WHICH WE WOULD TAKE A DUAL APPROACH TO GETTING IT THROUGH TO THE END OF THAT GRANTING PERIOD.

MR. SHEEHY: I'M JUST TRYING TO UNDERSTAND THIS. AND I WONDER IF THIS MIGHT NOT HAVE BEEN SOMETHING THAT WE SHOULD HAVE HEARD IN COMMITTEE. BECAUSE THERE'S A DIFFERENCE BETWEEN ONE PROJECT WITH A COMPANY. THERE'S AN INTEREST THAT'S BEEN NEGOTIATED WITH THE INVESTIGATOR. AND THEN CO-FUNDING A WHOLE BASKET OF APPLICATIONS. LET'S SAY THAT THERE'S A COMPANY THAT HAS AN INTEREST IN A PARTICULAR DISEASE INDICATION. THEY COME IN, THEY

FUND FOUR OR FIVE DIFFERENT INVESTIGATORS, PICK THE BEST ONE AND SHELF THE REST. THOSE OTHER FOLKS, WHAT HAPPENS TO THEM? MAYBE STEVE MIGHT HAVE SOME QUESTIONS ON THIS.

DR. JUELSGAARD: SO IN MY EXPERIENCE, THERE ARE THREE PARTIES, AS I UNDERSTAND IT, INVOLVED IN WHAT YOU'RE PROPOSING. SO ONE IS THE INVENTOR OF THE PROJECT, THE DEVELOPER, WHETHER IT'S A RESEARCH INSTITUTION OR A COMPANY OR WHATEVER IT IS, AND THEN THERE'S A BIGGER COMPANY, WHETHER IT'S 500 MILLION OR SOME OTHER NUMBER, IT COULD BE ANOTHER POINT OF DISCUSSION, AND THEN THERE'S CIRM. AND SO CIRM IS POTENTIALLY PROVIDING MONEY.

BUT IN THE VERY FIRST INSTANCE, THE COMPANY THAT HAS THE PROJECT THAT'S SEEKING A PARTNERSHIP WITH A BIGGER COMPANY HAS EVERY INCENTIVE NOT TO HAVE HAPPEN WHAT IT IS THAT YOU JUST TALKED ABOUT. AND IT'S VERY TYPICAL, IN FACT, IT WOULD BE ATYPICAL NOT TO, TO HAVE A CLAW-BACK PROVISION. IN OTHER WORDS, IF YOU DECIDE TO STOP WORKING ON THIS, WE GET IT BACK. BECAUSE YOU DON'T WANT TO BE LEFT IN THAT POSITION HAVING BROUGHT THE BABY SO FAR FORWARD TO HAVE IT PUT ON THE SHELF. YOU WANT TO BE ABLE TO PULL IT BACK AND FIND SOMEBODY ELSE TO WORK ON IT OR WORK ON IT YOURSELF.

AND INVARIABLY THOSE FIND THEIR WAY INTO AGREEMENTS.

I'D ASK ANNE-MARIE IF SHE AGREES WITH THAT BECAUSE THAT'S CERTAINLY BEEN MY EXPERIENCE. SO I THINK THAT'S FIRST AND FOREMOST, THE BULWARK AGAINST WHAT YOU'RE CONCERNED ABOUT.

WHAT WE SHOULD BE ABLE TO DO IS REVIEW THAT AGREEMENT BEFORE WE MAKE A CO-FUNDING DECISION HERE TO MAKE SURE THAT THERE IS AN APPROPRIATE PROVISION THAT REALLY PREVENTS WHAT YOU'RE TALKING ABOUT FROM HAPPENING THAN HAVING SOME LARGE COMPANY JUST DECIDE THAT IT DOESN'T WANT TO CONTINUE WITH THIS AND THAT IT WILL JUST PUT IT ON THE SHELF BECAUSE IT HAS THE ABILITY TO AND NOTHING ELSE CAN BE DONE WITH IT. I THINK WE CAN DEAL WITH THAT.

MR. SHEEHY: BUT WHAT ABOUT A COMPANY THAT WANTS TO -- YOU'RE TALKING ABOUT OVER 500 MILLION, SO POTENTIALLY COMPANIES THAT COULD GO BUY TECHNOLOGY AND JUST SHOVE IT OFF. THEY WOULD HAVE AN INTEREST. I'M NOT SURE THAT THESE WOULD ALWAYS BE SOPHISTICATED, EARLY COMPANY STAGE. WE'RE TALKING ABOUT INVESTIGATORS. SO LET'S SAY YOU SEE POTENTIAL COMPETITORS. YOU MIGHT PICK THEM UP AND SHELVE THEM SO THEY DON'T COMPETE WITH YOUR PRODUCT.

MS. BAUM: I'D ALSO LIKE TO MENTION THAT OUR IP REGULATIONS ALSO ADDRESS THIS BECAUSE IN OUR

IP REGULATIONS WE ALLOW UNIVERSITIES, NON-PROFITS, TO ENTER INTO EXCLUSIVE LICENSES. AND IT HAS A WHOLE SET OF CAVEATS AND PRECAUTIONS THAT PROTECT THE PROJECT, MEANING THAT THERE HAS BEEN A DEVELOPMENT PROGRAM THAT THE PARTIES AGREE TO, THERE NEEDS TO BE REMEDIES IN THE INSTANCE WHERE THE PROGRAM IS NOT PROGRESSING ETC., ETC. AND, OF COURSE, THE CLAW-BACK PROVISIONS WHICH WILL ALWAYS BE THERE.

AND, STEVE, I THINK THAT'S GREAT. WE SHOULD PUT IN A CLAUSE THAT WE GET TO REVIEW THE CONTRACTS. WE GET TO REVIEW THE IP CONTRACTS IN THE CASE OF THE COLLABORATIVE FUNDING PARTNERS AGREEMENTS THAT WE DO WITH OUR GOVERNMENT ENTITIES. SO WE'RE USED TO DOING THAT.

DR. JUELSGAARD: JUST TO ANSWER JEFF'S QUESTION ONE MORE TIME. SO, JEFF, THE INSTITUTIONS THAT WE'RE DEALING WITH THAT ARE CARRYING OUT THESE PROJECTS FOR THE MOST PART, THESE ARE NOT THE ONLY THINGS THEY DO IN THEIR LIFE. THEY ARE INVOLVED WITH A LOT OF OTHER PROJECTS AS WELL, WHICH I IMAGINE GET LICENSED WHOLLY INDEPENDENTLY OF CIRM IN OTHER SITUATIONS THAT HAVE NOTHING TO DO WITH REGENERATIVE MEDICINE. IT MIGHT HAVE TO DO WITH THE ANTIBODY WORLD OR GOD KNOWS WHAT OR EVEN NONMEDICAL

USES.

SO IT SEEMS TO ME THAT THEY HAVE BUILT INTO THEIR INSTITUTIONAL MECHANISMS WAYS OF DEALING WITH THE ISSUE THAT THEIR RESEARCH WOULD JUST BE SIDELINED SOMEWHERE BY SOME COMMERCIAL ENTERPRISE. SO I WOULD BE SURPRISED IF MOST OF THESE ORGANIZATIONS THAT DO THIS KIND OF WORK ACCOUNT FOR THAT WHEN THEY ENTER INTO AGREEMENTS WITH OTHERS.

BUT I HAVE ANOTHER QUESTION.

CHAIRMAN THOMAS: YOU ASK THAT QUESTION, THEN DEAN HAWGOOD.

DR. HAWGOOD: I WAS JUST GOING TO BUILD ON WHAT STEVE WAS SAYING, JEFF. I THINK HE'S EXACTLY RIGHT. WE HAVE ANY NUMBER OF EXACTLY THOSE KIND OF TERMS BUILT INTO MANY OF OUR INDUSTRY AGREEMENTS SUCH THAT IF OUR PARTNER DOES NOT WANT TO PROCEED FOR WHATEVER REASON, THEN IT REVERTS BACK. AND THAT'S CONTRACTUALLY BUILT IN.

DR. JUELSGAARD: SO, ELONA, QUESTION FOR YOU. SO YOU REFERRED TO A COLLABORATION AND CO-FUNDING AGREEMENT IN THIS PRESENTATION. COULD YOU JUST OUTLINE QUICKLY THE OBLIGATIONS OF CIRM IN SUCH AN AGREEMENT AND THE OBLIGATION OF THE COMPANY THAT'S A PARTY TO THIS? WHAT ARE THEIR OBLIGATIONS UNDER THIS AGREEMENT?

MS. BAUM: WELL, IT'S VERY LIGHT, I WOULD SAY, IN TERMS OF THE OBLIGATIONS OF CIRM. SO CIRM'S OBLIGATIONS ARE TO PROVIDE SOME SORT OF NOTIFICATION, THE INTEREST OF THE PARTIES OF THE FUNDING ENTITY, IN SUPPORTING CIRM-FUNDED RESEARCH EITHER ON OUR WEB SITE AND/OR WITHIN THE ACTUAL RFA THAT'S POSTED. THAT'S THE FIRST ONE.

AND THEN THE SECOND ONE WOULD BE MAYBE TO MAKE SOME EFFORT TO CREATE RESEARCH TEAMS, BUT MY FIRST DRAFT AT THIS WAS VERY LOOSE, AND IT DIDN'T REQUIRE AN ABSOLUTE REQUIREMENT TO DO MEETINGS AND LINKAGES.

THE OTHER MORE SIGNIFICANT ONE THAT I MENTIONED IS THAT WE DO GIVE THEM THE OPPORTUNITY TO PROVIDE US INPUT INTO THE CONCEPT PLAN FOR THE RFA AND THAT'S IT. VERY CAREFUL THIS DRAFTING OF A SAMPLE AGREEMENT NOT TO COMMIT ANYTHING ELSE.

DR. JUELSGAARD: WHAT ARE THEIR OBLIGATIONS?

MS. BAUM: THEIR OBLIGATIONS WOULD BE TO CO-FUND AS REQUIRED BY THE RFA OR AT LEAST TO PROVIDE SOME SORT OF FUNDING TO THE ACTUAL GRANTEE TO ENABLE THAT GRANTEE TO MAKE ITS MATCH AS REQUIRED BY THE RFA. SO WE WILL HAVE TO OVERSEE THAT THAT ACTUALLY OCCURS. AND THEN IT WAS TO MAKE SURE THAT

THEY CONTINUE TO PROVIDE THAT CO-FUNDING IF MILESTONES ARE MET, AND THERE IS A PROVISION THAT WE CAN ADDRESS A FAILURE TO MEET MILESTONES BECAUSE YOU CAN ENVISION, AS WHAT HAPPENS WITH THE CFP SITUATION, WHERE A MILESTONE IS NOT MET AND THEN THE TWO PARTIES WANT TO DO SOMETHING DIFFERENT. CIRM MIGHT WANT TO CONTINUE AND THE OTHER PARTY MIGHT NOT. AND THEN WE PROVIDE SORT OF A MECHANISM TO UNWIND.

BUT THAT IS ALL THE DETAIL. WHAT WE'RE TRYING TO FOCUS ON NOW IS REALLY JUST THE PROGRAM ANNOUNCEMENT. AND WHAT THE DETAILS OF THE CONTRACT ULTIMATELY WILL BE WE CAN DISCUSS LATER.

DR. JUELSGAARD: I AGREE WITH THAT. I THINK MAYBE JUST DROPPING THE WORD "AGREEMENT" OUT OF ALL THIS ANNOUNCEMENT AT THIS POINT MIGHT BE A GOOD THING TO DO. I'M NOT SURE YOU HAVE TO HAVE AN AGREEMENT TO DO ALL THE THINGS THAT YOU'RE TALKING ABOUT AT THIS POINT.

LASTLY, YOU HAVE THIS NOTION OF 50-50 COST SHARING. AND THIS ACTUALLY GOES TO DISCUSSIONS THAT WE HAD OTHERWISE ABOUT FLEXIBILITY IN THESE ARRANGEMENTS. SO WHAT IF SOMEBODY SAID, WELL, I'M WILLING TO CO-FUND, BUT I'D LIKE CIRM TO CO-FUND 60 AND I'LL CO-FUND 40? SO ARE WE GOING TO WRITE THAT

OFF BECAUSE IT'S GOT TO BE 50-50 OR NOTHING AT ALL?

MS. BAUM: WELL, I PERSONALLY AM ONE WHO LIKES FLEXIBILITY. I THINK WHAT IT WOULD DEPEND ON IS HOW WE END UP DRAFTING THE RFA WHICH COULD BE FLEXIBLE. RIGHT NOW WHAT WE TRY TO SAY IS THAT YOU CAN MAKE UP SOME OF THE REQUIRED MATCH WITH IN-KIND SERVICES. AND WE FEEL LIKE WE STRUCK A FLEXIBLE GROUND THAT IS APPEALING TO THE PHARMAS WITH THAT.

THE PROBLEM IS IT'S KIND OF LIKE A GAME OF CHICKEN. YOU HAVE TO SORT OF PUSH THIS ON PEOPLE OR ELSE THEY MIGHT NOT END UP GENERATING THE INTEREST. BUT THERE IS ALWAYS THE EXCEPTIONS PATHWAY THAT PROVIDES US FLEXIBILITY UNDER THE RFA. SO WE TRY TO GO OUT PRETTY STRONG WITH WHAT OUR DESIRE IS. WE HAVE AN IN-KIND SERVICES APPROACH TO MEETING PART OR ALL OF THAT, AND THEN THERE'S AN EXCEPTIONS PATHWAY.

CHAIRMAN THOMAS: I MAY HAVE MISSED THIS, ELONA. DID YOU ANSWER MR. SHEEHY'S QUESTION ABOUT ARE THERE ANY COMPANIES INTERESTED?

MS. BAUM: NO, I DID NOT BECAUSE WE TOOK A DIFFERENT TURN. SO THERE WAS ONE COMPANY THAT HAS EXPRESSED CONSIDERABLE INTEREST, AND IT WAS SOME TIME AGO. SO I HOPE THEY'RE STILL INTERESTED. I'M NOT SURE.

MR. SHEEHY: CAN WE SAY WHO THEY ARE?

MS. BAUM: I DON'T THINK I SHOULD STATE WHO THEY ARE.

MR. SHEEHY: I MEAN AT WHAT POINT DO WE HAVE, LIKE, SOME SORT OF TRANSPARENCY? WE ARE A STATE AGENCY. WE'RE TALKING ABOUT BEING PARTNERS WITH SOMEBODY.

MS. BAUM: THE WHOLE POINT OF THIS IS TO MAKE IT VERY TRANSPARENT BECAUSE WE WANT TO, AS SOON AS IT'S EXECUTED, ADVERTISE IT SO THAT THE CALIFORNIA RESEARCHERS KNOW WHO TO ENGAGE. SO, OF COURSE, AS SOON AS IT IS EXECUTED, WE WILL MAKE THIS VERY VISIBLE. AND, OF COURSE, WE'LL DO EVERYTHING WE CAN TO GET MORE PEOPLE INTERESTED.

DR. TROUNSON: JEFF, I THINK IT'S JUST THAT WE DON'T WANT TO SAY NOW BECAUSE WE HAVEN'T GOT ANY AGREEMENT WITH THEM. I DON'T THINK WE'VE PROGRESSED IT THAT DISTANCE. OF COURSE, WE WOULD HAVE TO LIST IT ON THE WEB SITE AND EVERYTHING ELSE. IT WOULD HAVE TO BE TRANSPARENT, BUT RIGHT NOW WE HAVEN'T TAKEN THE DISCUSSIONS FAR ENOUGH TO KNOW WHETHER THEY'D STILL BE INTERESTED.

MR. SHEEHY: I GUESS I'M JUST KIND OF UNCLEAR ABOUT HOW ALL THIS IS GOING TO WORK. I FEEL LIKE I'M VOTING NOW TO BECOME PARTNERS WITH COMPANIES, NAMES OF WHICH I DO NOT KNOW, AND I FEEL

VERY UNCOMFORTABLE ABOUT THAT. I DON'T NECESSARILY THINK CONCEPTUALLY THIS IS A BAD IDEA, BUT I ALMOST FEEL LIKE IT'D BE BETTER TO HAVE A WORKSHOP AND SAY THIS IS WHAT WE'RE TALKING ABOUT DOING, GETTING A SENSE OF WHO'S INTERESTED AND HOW THIS WOULD PROCEED. IT JUST SEEMS -- IT'S ONE THING TO MAKE ARRANGEMENTS WITH GOVERNMENTS, WITH NONPROFIT AGENCIES; BUT I WONDER, WHEN YOU'RE TALKING ABOUT HEAVILY CAPITALIZED COMPANIES, WHAT THAT RELATIONSHIP IS REALLY GOING TO LOOK LIKE AND HOW WE BALANCE OUR INTERESTS WITH THEIR INTERESTS.

DR. DULIEGE: JEFF, AREN'T WE VOTING TODAY FOR A PROPOSAL AND A PROCESS IN GENERAL INDEPENDENTLY OF WHICH COMPANY HAS ALREADY MENTIONED INTEREST OR WILL IN THE FUTURE?

MR. SHEEHY: I'M NOT SURE WHAT WE'RE VOTING ON. WE HAVE A CONCEPT. I DON'T KNOW WHAT THE PRICE TAG IS ASSOCIATED WITH THIS, BUT CERTAINLY WE ARE TALKING ABOUT SETTING ASIDE SOME SEGMENT OF MONEY TO DO THIS.

MS. BAUM: I'LL ANSWER THAT. WHAT WAS ENVISIONED IS THIS WOULD REALLY JUST SORT OF FACILITATE WHAT IS ALREADY SET ASIDE FOR STRATEGIC PARTNERSHIP FUNDS. SO, AS I SAID, THIS IS JUST TRYING TO JUMP-START THOSE NEGOTIATIONS THAT ARE

REQUIRED TO CREATE THE ELIGIBILITY REQUIREMENT FOR COMPANIES. SO THEY COULD ACTUALLY GO TO SOME OF THESE COMPANIES ON THEIR OWN, THE ONES THAT WOULD BE POTENTIAL CO-FUNDERS, AND COME IN IF THEY WERE ABLE TO STRIKE A DEAL. BUT I'D RATHER START THE PROCESS EARLIER IN THE NEGOTIATIONS WITH THESE LARGER COMPANIES AND THE CALIFORNIANS.

DR. WESTON: WHAT HAPPENS ONCE THERE'S A DISCOVERY?

MS. BAUM: I DIDN'T HEAR YOU.

DR. WESTON: THESE ARE VERY EARLY STAGE PROJECTS OR ALONG THE WAY TO TRANSLATION, BUT THERE'S NO DISCOVERY YET. SO WHAT HAPPENS ONCE THERE IS A DISCOVERY?

MS. BAUM: I THINK THERE'S A MISUNDERSTANDING HERE. STRATEGIC PARTNERSHIP FUNDING RFA'S HAVE ALWAYS BEEN IN THE LATTER STATE OF THE DEVELOPMENT. THEY ARE IN THE DEVELOPMENT PIPELINE. SO THERE WILL BE A CANDIDATE THAT'S REQUIRED, A DRUG CANDIDATE.

DR. WESTON: THEN HOW WILL YOU SHARE THE COST OF THE DEVELOPMENT OF THAT 50-50 BECAUSE THOSE CAN BE INCREDIBLY EXPENSIVE?

MS. BAUM: SO THE STRATEGIC FUNDING PARTNERSHIP PROGRAM HAS TRADITIONALLY BEEN TEN FROM

CIRM OR UP TO TEN FROM CIRM AND A MATCH BY THE GRANTEE FOR USUALLY A COMPLETION OF A PHASE I AND/OR A PHASE II.

DR. WESTON: SO THE COST OF THE DEVELOPMENT IS CAPPED SOMEWHERE. IT'S NOT THROUGH TO FDA APPROVAL. IT'S SOME EARLIER STAGE?

MS. BAUM: THE WAY OUR RFA'S WORK, THAT'S CORRECT.

MR. SHEEHY: AREN'T RFA'S BASED ON CONCEPTS THAT THEY WOULD BE INTIMATELY INVOLVED IN DRAFTING?

MS. BAUM: THEY HAVE THE OPPORTUNITY TO PROVIDE INPUT. WE CAN ACCEPT OR REJECT ANY INPUT THAT WE RECEIVE.

MR. SHEEHY: THE PROBLEM WE'RE TRYING TO ADDRESS IS THAT WE HAVE A STRATEGIC PARTNERSHIP PROGRAM IN WHICH THE PEOPLE WHO CAN'T GET APPLICATIONS BECAUSE PEOPLE CAN'T FIND CO-FUNDING FOR THEM?

MS. BAUM: RIGHT. AND THEY ARE ENTERING NEGOTIATIONS WITH VARIOUS PHARMA AND BIOPHARMA, BUT THOSE TAKE TIME. SO WE FIND THAT WE'RE GOING OR WE'RE IN THE PLACE WHERE, GEE, THEY HAVE A LETTER OF INTEREST, IT'S NOT VERY SOLID, DO WE WANT TO INVEST RESOURCES FOR GWG REVIEW? THEN DO WE WANT TO HAVE A

TEAM THAT SCORES HIGH, BUT HASN'T ACTUALLY SECURED A FINAL AGREEMENT BE BEFORE THE ICOC? SO WHAT WE'RE TRYING TO DO IS JUMP-START THE TIMING WITH THE CREATION OF POTENTIAL PROGRAMS AND RELATIONSHIPS EARLIER ON.

DR. WESTON: SORRY. I JUST DON'T QUITE UNDERSTAND THE PROCESS. SO YOU WOULD COME BACK HERE WHEN YOU HAVE AN AGREEMENT THAT YOU WOULD WANT THE BOARD TO APPROVE ONCE YOUR LETTER OF INTENT IS SIGNED, AND THAT WOULD HAVE THE CAP OF MONEY THAT THE STATE IS COMMITTING TO OR CIRM IS COMMITTING TO?

MS. BAUM: WHAT WE WOULD DO, AS WE ALWAYS DO, WE'D COME BACK TO THE BOARD WHEN WE HAVE A SCORE FROM A GWG THAT SAYS, YES, WE RECOMMEND APPROVAL FOR THIS STRATEGIC PARTNERSHIP-FUNDED PROGRAM IN THE AMOUNT OF UP TO $10 MILLION. WE WILL NOT BE COMING WITH THE ACTUAL AGREEMENT.

MR. SHEEHY: BUT WHAT IF THE COMPANY BACKS OUT?

MS. BAUM: WELL, THAT WOULD BE THE RISK WITH OR WITHOUT THIS PROGRAM. IT'S ALWAYS A RISK. AND IF IT BACKS OUT, OF COURSE, THERE WILL BE THE CLAW-BACK PROVISIONS. AND THEN THE QUESTION IS WOULD THE GRANTEE HAVE THE MONEY TO CONDUCT THE REST OF THE TRIAL? THERE WILL BE PROVISIONS THAT ADDRESS

THIS WITHIN ANY AGREEMENT. THIS IS NO DIFFERENT THAN WHAT WE WOULD HAVE RIGHT NOW.

MS. SAMUELSON: TELL ME IF THIS IS WHAT THE SITUATION IS, THAT WE'RE TRYING TO ATTRACT INDUSTRY FUNDING, INDUSTRY INVOLVEMENT IN A BIOMEDICAL DEVELOPMENT. AND THIS WOULD PROVIDE A FRAMEWORK THAT WOULD JUMP-START, AS YOU SAID.

MY PERCEPTION OF WHAT THE PROBLEM IS IS THE SCIENCE ISN'T DEVELOPED ENOUGH FOR INDUSTRY TO SEE IT AS ENOUGH OF A WIN BECAUSE THE RISK IS STILL TOO HIGH. AM I WRONG?

MS. BAUM: WELL, I WOULD SAY THAT'S NOT PRECISE. I THINK THAT IT DEPENDS ON WHO THE ORGANIZATION IS. OBVIOUSLY THERE AREN'T MANY BIOPHARMAS THAT ARE FUNDING IN THIS AREA, BUT THERE IS NOT A ZERO. THERE HAVE BEEN THE PFIZERS WHO HAVE INVESTED. THERE'S J & J THAT ACTUALLY INVESTED -- AT LEAST J & J DEVELOPMENT CORP. THAT JUST INVESTED IN VIACYTE. SO, YES, THERE ARE SOME OUT THERE. THERE'S THIS ONE ORGANIZATION THAT HAS EXPRESSED SOME INTEREST. SO WHILE IT IS A YOUNG FIELD AND SOME COMPANIES ARE CAUTIOUS, NOT ALL ARE.

MS. SAMUELSON: THEY MOVED AHEAD BECAUSE THEY HAVE THIS SORT OF FRAMEWORK?

MS. BAUM: WELL, I WOULD ABSOLUTELY SAY

THAT THE FUNDING THAT VIACYTE RECEIVED FROM ITS PARTNERS, INCLUDING J & J, WOULD NOT HAVE HAPPENED BUT FOR STRATEGIC PARTNERSHIP BECAUSE WE HAD PROVIDED THEM WITH $10 MILLION AND SAID THEY HAD TO HAVE A MATCH. AND SO THEY WENT OUT AND FOUND THAT MATCH.

DR. TROUNSON: WE ARE GETTING INCREASING INTEREST FROM THE BIOPHARMACEUTICAL INDUSTRY, AND SOME OF THOSE PROJECTS ARE EARLY AND SOME OF THEM, ONE BY ONE THEY'RE COMING IN TO SAY WE'D LIKE TO CO-FUND THAT. AND IT CAN BE AS EARLY AS THE EARLY TRANSLATION, AND IT CAN BE AS LATE AS THE DISEASE TEAMS GOING TO THE CLINIC. SO THERE'S MORE AND MORE INTEREST. AND SO SOME OF THE COMPANIES ARE PROBABLY INTERESTED IN TWO OR THREE PROJECTS, MAYBE FOUR OF THEM, BUT IT'S A RESTRICTED NUMBER. BUT THERE'S AT LEAST THE ODD COMPANY. THERE'S THE LARGEST COMPANIES WHO SEE THEY WOULD LIKE TO EXPAND THEIR PORTFOLIO OF INTEREST, AND THIS IS ONE WAY OF COMING IN WITH US AND TO EXPAND THEIR INTEREST WITH THE POSSIBILITY THAT THEY COULD FUND HOPEFULLY UP TO TEN PROJECTS OVER SEVERAL YEARS.

AND I THINK FOR US WE NEED TO KEEP THINKING ABOUT THESE THINGS BECAUSE IN THE EVENT THAT WE DON'T GET RE-FUNDED AT ALL, WE'RE GOING TO

HAVE A LOT OF THESE PROJECTS ORPHANED, IF YOU LIKE, WITHOUT PEOPLE TO LOOK AFTER THEM. SO MY CONCERN AT THE MOMENT, I THINK, IS ABOUT MAKING SURE THAT WE CREATE ALL THE OPPORTUNITIES WE CAN TO LEVERAGE WHAT DOLLARS THAT WE'VE GOT CURRENTLY IN THE BANK ACCOUNT WITH THOSE TRANSLATIONAL PROJECTS. THEY'RE ALL TRANSLATION GOING TO THE CLINIC. AND TO SEE IF WE CAN HELP AS MANY OF THEM AS WE CAN IN THE TIME FRAME THAT WE HAVE.

OF COURSE, IF WE GET A NEW PROPOSITION OR NEW MONEY COME IN, IT MAKES IT A LOT EASIER FOR US. BUT CURRENTLY WE ARE AT THAT EDGE THAT WE COULDN'T PROBABLY TAKE MORE THAN ABOUT 15 OR 20 PERCENT AT THE MOST OF WHAT WE'VE GOT THROUGH TO EVEN TO PHASE II.

MR. SHEEHY: YOU KNOW, I WONDER -- I MEAN THIS IS AN INTERESTING IDEA. I PERSONALLY DON'T KNOW THAT THIS WOULD BE SOMETHING THAT I COULD SUPPORT AT THIS TIME. I WONDER IF IT WOULD MAKE SENSE TO REFER THIS TO THE IP AND INDUSTRY SUBCOMMITTEE AND GET SOME OF THESE DETAILS REALLY KIND OF WORKED OUT.

MR. TORRES: SECOND THAT.

MR. SHEEHY: I DON'T KNOW IF STEVE THINKS HE'D BE COMFORTABLE KIND OF CONDUCTING THAT KIND OF.

I JUST WOULD -- I JUST DON'T KNOW. I JUST FEEL LIKE IT NEEDS A LITTLE MORE. I DON'T KNOW.

DR. DULIEGE: JEFF, WE CERTAINLY CAN DO THAT IF YOU WANT TO. BUT IF I UNDERSTAND CORRECTLY, WHAT THIS PROPOSAL IS ALL ABOUT IS SIMPLY HELPING GRANTEES THAT HAVE SHOWN SOME SCIENTIFIC MERIT GET THE SUPPORT OF THE CIRM TO GET INDUSTRY FUNDING OR WHICHEVER THIRD-PARTY FUNDING THEY NEED TO MATCH THE PROPOSAL, IN THAT CASE BEING INDUSTRY. SO THAT'S ACTUALLY A VERY SIMPLE CONCEPT. AND FOR ANY TYPE OF CONTRACT THAT WE'RE HAVING WITH THE THIRD PARTY, THERE ARE PROVISIONS FOR IF THEY FAIL TO -- NOT IN THE SITUATION TO DELIVER ON THEIR COMMITMENTS. THAT'S PART OF ANY NEGOTIATION, ANY CONTRACT.

EXAMPLE. A PARTY COMMIT TO $10 MILLION, BUT THEY COMMIT AS PER MILESTONES BEING REACHED. IF THESE MILESTONES ARE NOT REACHED, THEY'RE OBLIGATED TO PAY. THEY'RE OBLIGATED TO PAY UNLESS THEY BECOME INSOLVABLE, WHICH IS A VERY DIFFERENT SITUATION. AND HAVING A BOTTOM FLOOR OF $500 MILLION LIMITS THE RISK OF THESE COMPANIES BECOMING ONE DAY INSOLVABLE.

SO WE CAN DEFINITELY BRING IT BACK TO THE IP SUBCOMMITTEE. I'LL TELL YOU THIS IS THE KIND OF DATA WE'LL HAVE OVER. FOR ME IT'S A LITTLE BIT OF A NO BRAINER I HAVE TO SAY, AND I WOULD VOTE FOR IT

TODAY.

CHAIRMAN THOMAS: MR. JUELSGAARD, WHAT'S YOUR STATE OF PLAY OPINION?

DR. JUELSGAARD: WELL, I THINK THE ULTIMATE GOAL HERE IS TO TRY AND ATTRACT INDUSTRY TO LOOK AT OUR PROJECTS AND HOPEFULLY TAKE SOME OF THEM ON. AND THE QUESTION IS WHAT'S THE BEST WAY TO DO THAT. AND I THINK THAT'S WHAT ELONA AND ALAN HAVE BEEN SEARCHING FOR, AND THIS IS ONE OF THE POSSIBLE WAYS OF DOING THAT. I THINK IT ACTUALLY MIGHT BE A GOOD THING AT AN UPCOMING IP AND INDUSTRY SUBCOMMITTEE MEETING JUST TO TAKE ON THAT SUBJECT AND LOOK AT VARIOUS WAYS GIVEN THE DIFFERENT EXPERIENCES THAT PEOPLE ON THAT COMMITTEE HAVE HAD OF HOW YOU TRY TO ENTICE INDUSTRY TO COME ALONG FURTHER THAN THEY HAVE TO DATE WITH THE SORT OF PROJECTS THAT WE HAVE.

BUT ALONG WITH ANNE-MARIE, I THINK THAT I DON'T KNOW THAT THIS WILL BE THAT SUCCESSFUL. UNTIL WE TRY IT, WE DON'T KNOW. BUT I DON'T SEE A LOT OF DOWNSIDE IN IT.

DR. HAWGOOD: I AGREE. IF I UNDERSTAND WHAT'S BEING PROPOSED, WE'RE SIMPLY PUTTING OUT A CALL FOR COMPANIES TO SELF-IDENTIFY WHO MIGHT BE INTERESTED IN ENTERING INTO ONE OF OUR REGULAR

FORMAL RFP-TYPE PROCESSES IN THE FUTURE. WE'RE NOT CALLING THIS AN AGREEMENT OR SEEMS TO HAVE MADE IT SOUND MORE COMPLEX THAN SIMPLY A CALL FOR COMPANIES TO SELF-IDENTIFY THAT THEY MIGHT BE INTERESTED IN WORKING WITH CIRM IN THE FUTURE. IS THAT RIGHT?

MS. BAUM: WELL, WE COULD CERTAINLY GO THAT ROUTE. I WAS HOPING TO ALSO GET AUTHORITY TO ENTER INTO AN AGREEMENT WITH THEM RATHER THAN JUST HAVE THEM SELF-IDENTIFY SO WE COULD TACKLE THE VARIOUS ISSUES THAT ACTUALLY WERE BROUGHT UP IN THE COURSE OF DISCUSSION.

DR. HAWGOOD: WHAT WOULD THEY BE AGREEING TO AT THIS POINT BECAUSE THE ACTUAL PROJECT HASN'T BEEN IDENTIFIED?

MS. BAUM: A PROCESS THAT WOULD DEAL WITH RIGHTS TO TERMINATE AND COMMITMENTS TO FUNDING WHICH ARE ALWAYS IN AGREEMENTS.

DR. HAWGOOD: SOME OF THE TECHNICAL ISSUES.

MS. BAUM: CONFIDENTIALITY, LETTING THEM KNOW THAT THEY AREN'T GOING TO BE IN THE GWG. IT'S SETTING FORTH AN AGREEMENT AROUND A PROCESS REALLY. THE REAL MEAT AND POTATOES IN ANY AGREEMENT WILL BE THE AGREEMENT THAT'S BETWEEN THE GRANTEE AND THE FUNDING PARTNER. THAT'S WHERE THE LICENSING AND THE

ROYALTY PROVISIONS WOULD BE.

DR. HAWGOOD: SO REALLY JUST HAVING ANY COMPANY THAT DOES SELF-IDENTIFY UNDERSTAND AT A VERY HIGH LEVEL THE RULES OF THE ROAD --

MS. BAUM: EXACTLY.

DR. HAWGOOD: -- THAT THEY WOULD BE AGREEING TO EVENTUALLY.

MS. BAUM: RIGHT. THEY WOULDN'T EVEN BE ENTITLED TO RECEIVE FUNDING UNLESS AND UNTIL THEY ENTER INTO AN AGREEMENT THAT'S SATISFACTORY TO CIRM WITH THE ACTUAL GRANTEE AND APPLICANT. IT'S VERY HIGH LEVEL.

CHAIRMAN THOMAS: ANNE-MARIE.

DR. DULIEGE: I THINK THIS IS ANOTHER TYPICAL EXAMPLE WHERE I RECOMMEND THAT WE LET CIRM MAKE THESE DECISIONS, MOVE AHEAD, AND THAT THE BOARD SHOULD -- WE SHOULD DISTANCE OURSELF A LITTLE BIT FROM THE NITTY-GRITTY. IF THERE ARE QUESTIONS, CIRM WILL BE THE FIRST ONE TO COME AND ASK RECOMMENDATIONS OR HELP FROM THE BOARD, BUT THAT'S CIRM PRIVILEGE AND ACTUALLY PART OF THEIR ACCOUNTABILITY TO DO THAT.

MS. SAMUELSON: IT WOULDN'T HURT THOUGH, I THINK, TO GET THE INPUT OF THE IP SUBCOMMITTEE BEFORE THIS FINALLY BEGINS BECAUSE WE HAVE HAD

EXPERIENCE WITH PUTTING UP A LOT OF MONEY TOWARD CLINICAL TRIALS AND THERE HAVE BEEN ABRUPT ENDINGS OF THEM, AND ALL OF THAT EFFORT WAS FOR NAUGHT. AND SO IF THIS IS NOT GOING -- IT'S -- WE'VE HAD A VERY DIFFICULT TIME GETTING TO THE CLINICAL TRIAL PHASE, RIGHT? AND I'M NOT SURE THAT THESE ARE THE REAL OBSTACLES AS OPPOSED TO THERE ISN'T SUFFICIENT SCIENCE TO CONVINCE THE INDUSTRY PARTNER WITHOUT SOME OTHER INCENTIVE LIKE SOME OF OUR MONEY OR A LOT OF OUR ATTENTION TO JOIN IN IN A PARTNERSHIP OF SOME KIND. IT ENDS UP SPINNING OUR WHEELS, AND WE REALLY NEED TO FOCUS ON THE MOST EFFECTIVE TOOLS. I THINK THERE'S SOME EXPERIENCE NOW TO APPLY TO IT.

MR. ROWLETT: WE ADDRESSED -- WE HAD A SECOND BY SENATOR TORRES, I THINK, TO TAKE A VOTE ON WHETHER OR NOT WE TAKE THIS TO THE -- WHAT WAS YOUR MOTION?

CHAIRMAN THOMAS: MR. HARRISON, IS THAT A VOTABLE TOPIC, REFERRING TO INDUSTRY AND IP SUBCOMMITTEE?

MR. HARRISON: IT IS.

MR. TORRES: I WOULDN'T HAVE MADE IT IF IT WASN'T.

CHAIRMAN THOMAS: YES, MR. SENATOR. OKAY. ANY FURTHER DISCUSSION ON THAT TOPIC BEFORE WE VOTE

ON THAT AS AN INTERIM? AND IF IT DOES GO, THEN OBVIOUSLY WE TABLE THE MOTION WRIT LARGE HERE. OKAY. MR. HARRISON, I ASSUME THIS COULD BE A VOICE VOTE?

MR. HARRISON: YES. AFTER PUBLIC COMMENT, A VOICE VOTE AND ROLL CALL VOTE FOR THE PHONE.

CHAIRMAN THOMAS: ANY PUBLIC COMMENT ON THIS TOPIC? COMMENTS FROM ANYBODY ON THE PHONE? ALL THOSE IN FAVOR OF REFERRING THIS TO IP AND INDUSTRY SUBCOMMITTEE IN ADVANCE OF BRINGING IT TO THE BOARD UPON THEIR DELIBERATIONS PLEASE SAY AYE. OPPOSED?

SOUNDED LIKE A BUNCH OF HORSES IN A BARN.

ROLL CALL VOTE. I THINK THAT THE AYES HAD IT, BUT WE'LL SEE.

MS. BONNEVILLE: SUE BRYANT.

CHAIRMAN THOMAS: AYE MEANS YOU'RE REFERRING IT TO THE IP AND INDUSTRY SUBCOMMITTEE.

DR. DULIEGE: NO MEANS IT WILL BE APPROVED DIRECTLY WITHOUT REFERRING BACK TO SUBCOMMITTEE?

CHAIRMAN THOMAS: CORRECT. IT HAS TO COME BACK HERE.

MR. HARRISON: SO THIS MOTION IS SOLELY FOR THE PURPOSE OF WHETHER THE BOARD WANTS TO FIRST REFER THE PROGRAM ANNOUNCEMENT TO THE IP AND

INDUSTRY SUBCOMMITTEE BEFORE COMING BACK TO THE BOARD WITH A RECOMMENDATION ABOUT WHETHER TO VOTE FOR OR AGAINST THE PROGRAM. IF THIS MOTION IS REJECTED, THEN THE BOARD COULD TAKE UP A MOTION TO APPROVE THE PROGRAM ANNOUNCEMENT AS IS.

CHAIRMAN THOMAS: THAT'S CORRECT.

MR. HARRISON: A YES VOTE HERE MEANS YOU WANT IT TO FIRST GO TO THE IP AND INDUSTRY SUBCOMMITTEE.

MS. BONNEVILLE: SUE BRYANT.

DR. BRYANT: NO.

MS. BONNEVILLE: KEN BURTIS.

DR. BURTIS: NO.

MS. BONNEVILLE: CARL WARE.

DR. WARE: NO.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DR. DULIEGE: NO.

MS. BONNEVILLE: MARCY FEIT. LEON FINE. ELIZABETH FINI.

DR. FINI: NO.

MS. BONNEVILLE: MICHAEL FRIEDMAN. MICHAEL GOLDBERG.

MR. GOLDBERG: YES.

MS. BONNEVILLE: SAM HAWGOOD.

DR. HAWGOOD: NO.

MS. BONNEVILLE: STEPHEN JUELSGAARD.
MR. JUELSGAARD: NO.
MS. BONNEVILLE: SHERRY LANSING. BERT LUBIN. LLOYD MINOR.
DR. MINOR: NO.
MS. BONNEVILLE: KIRK PETERSON.
DR. PETERSON: NO.
MS. BONNEVILLE: FRANCISCO PRIETO.
DR. PRIETO: AYE.
MS. BONNEVILLE: ROBERT QUINT.
DR. QUINT: YES.
MS. BONNEVILLE: AL ROWLETT.
MR. ROWLETT: YES.
MS. BONNEVILLE: JOAN SAMUELSON.
MS. SAMUELSON: YES.
MS. BONNEVILLE: JEFF SHEEHY.
MR. SHEEHY: YES.
MS. BONNEVILLE: OSWALD STEWARD. JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: EUGENE WASHINGTON. DONNA WESTON.
DR. WESTON: YES.

MS. BONNEVILLE: DIANE WINOKUR.

MS. WINOKUR: NO.

MR. HARRISON: SO THAT MOTION FAILS BY A VOTE OF NINE YES TO TEN NO.

CHAIRMAN THOMAS: VERY INTERESTING, MR. HARRISON. OKAY. SO THEN THAT HAVING BEEN SAID, I ASSUME -- IS THERE ANY FURTHER DISCUSSION ON THE ORIGINAL MOTION TO APPROVE -- THERE IS NO MOTION. IS THERE A MOTION TO APPROVE?

DR. HAWGOOD: SO MOVED.

DR. PETERSON: SECOND.

CHAIRMAN THOMAS: SECONDED BY KIRK PETERSON. OKAY. ANY FURTHER DISCUSSION?

DR. WESTON: IT DOES STRIKE ME THAT $500 MILLION IN CAPITALIZATION IS AN AWFULLY BIG NUMBER, AND I WONDER IF THERE'S ANOTHER WAY WITH SMALLER ORGANIZATIONS THAT YOU CAN CAUSE THEM TO SET ASIDE THEIR REQUISITE AMOUNT OF CAPITAL OR SOMETHING TO ALLOW MORE PLAYERS TO PARTICIPATE IN THIS ARENA.

MS. BAUM: WELL, RIGHT NOW THAT'S HOW OUR STRATEGIC PARTNERSHIP FUNDING RFA IS, BUT IT CAN CHANGE BY RFA, WHICH IS SUBJECT TO BOARD APPROVAL AT THE CONCEPT LEVEL.

CHAIRMAN THOMAS: OTHER COMMENTS? QUESTIONS?

MR. SHEEHY: I JUST REALLY THOUGHT THAT IT WOULD HAVE BEEN USEFUL TO REALLY TRY TO, INSTEAD OF CONTINUING -- WE APPROVE THIS LIKE THE FOURTH STRATEGIC PARTNERSHIP THING WE'VE DONE. AND RATHER -- IT JUST IT SEEMS TO ME THAT I WOULD HOPE THAT THE INDUSTRY SUBCOMMITTEE WOULD TAKE THIS ON AND ACTUALLY TRY TO GET TO A SOLUTION OR A SET OF SOLUTIONS. IT JUST SEEMS VERY REACTIVE. AND SO I DON'T -- WHAT'S THE PRODUCTIVITY OF OUR CURRENT STRATEGIC PARTNERSHIP PROGRAM? THAT SHOULD BE ANALYZED. WE'RE TALKING ABOUT CAPITAL LIMITS THAT MAY BE A PROBLEM. AND SO, FINE, IF YOU GUYS WANT TO VOTE ON THIS. THERE'S NOTHING AGENDAD FOR THE INDUSTRY SUBCOMMITTEE. YOU'RE NOT REALLY DEALING WITH THE PROBLEM. YOU MAY JUST HAVE GIVEN ANOTHER MECHANISM TO FAIL. IF THAT IS CONSIDERED A GREAT WAY IN WHICH TO TRY TO ADDRESS WHAT SEEMS TO ME TO BE A SERIOUS POLICY ISSUE, THEN GO AHEAD.

DR. JUELSGAARD: SO, JEFF, I THINK A LITTLE EARLIER I SAID I THOUGHT IT WOULD BE A GREAT IDEA TO ACTUALLY HAVE A MEETING OF THE COMMITTEE TO TALK ABOUT, DISCUSS THE WAYS THAT THIS ORGANIZATION CAN TRY TO INTERACT WITH THE LARGER INDUSTRY, AND I STILL BELIEVE THAT. AND SO I THINK WE'LL TRY AND SET UP A MEETING IN THE NEAR FUTURE TO DO THAT.

BUT THIS IS ONE WAY -- AGAIN, I THINK THE JURY IS REALLY OUT AS TO WHETHER IT'S GOING TO GAIN ANY TRACTION OR NOT. I DON'T KNOW. BUT I HEAR WHAT YOU'RE SAYING, AND I THINK WE SHOULD DO THAT. AND IN THE MEANTIME, I DON'T THINK THAT THERE'S ANY -- I DON'T SEE ANY PARTICULAR DOWNSIDE TO WHAT THEY'RE PROPOSING, AND MAYBE THERE'S SOME UPSIDE.

MR. SHEEHY: HOW ABOUT A FRIENDLY AMENDMENT BECAUSE I THINK THESE TEN-NINE VOTES ARE NOT BENEFICIAL TO THE BOARD IN GENERAL. COULD I PROPOSE A FRIENDLY AMENDMENT, THAT PERHAPS THAT YOU ACTUALLY DO LOOK AT WHAT WE'RE DOING IN STRATEGIC PARTNERSHIP, THAT YOU LOOK AT WHAT'S WORKING AND WHAT'S NOT WORKING, PERHAPS OBTAIN SOME FEEDBACK FROM THE COMPANIES AND FROM POTENTIAL GRANTEES SO THAT WE COULD ACTUALLY TRY TO SOLVE THE PROBLEM THAT WE'RE TRYING TO DO. THEN I WOULD BE HAPPY TO SUPPORT THIS, BUT IT'S MORE LIKE -- I FEEL LIKE EVERY MEETING WE GET ANOTHER STRATEGIC PARTNERSHIP THING, AND THOSE PARTNERSHIPS AREN'T REALLY COMING TO FRUITION. WOULD THAT BE ACCEPTABLE TO THE MAKER THE SECOND?

CHAIRMAN THOMAS: I GUESS THE QUESTION IS, JEFF, WHAT'S THE DIFFERENCE BETWEEN THAT AND JUST RESOLVING SEPARATE FROM THIS TO --

MR. SHEEHY: IT'S A FRIENDLY AMENDMENT, SO IT WOULD GO ALONG WITH APPROVING THIS PROGRAM, BUT THAT WOULD ALLOW ME TO SUPPORT GOING AHEAD BECAUSE AT LEAST WE'D BE DOING SOME ANALYSIS INSTEAD OF CONTINUING TO APPROVE THINGS ENDLESSLY.

DR. TROUNSON: SO, CHAIR, WE'D BE HAPPY TO REPORT ON THAT TO THE SUBCOMMITTEE. AND WE'VE ONLY TWO OF THEM. THE THIRD ONE WE RECEIVED THE APPLICATIONS CURRENTLY. SO WE CAN COME BACK WITH THAT DATA. AND IT'S FAIRLY NEW, SO WE CAN REPORT ON HOW FAR WE'VE GOT WITH THOSE. AND IT IS A BIT INTERESTING. I'D SAY ABOUT THAT IT'S BEEN OPEN TO THE INDUSTRY EVERY SIX MONTHS, AND THEY'VE GOT A BIG, REASONABLY BIG RESPONSE THIS TIME AND NOT MUCH LAST TIME. AND IT COMES A BIT IN WAVES, SO WE MAY HAVE TO LOOK OVER A LONGER TERM INTO THE WHOLE PROGRAM, BUT WE'RE HAPPY TO DO THAT NOW, GIVE YOU SOME INFORMATION THAT YOU CAN UTILIZE. NO PROBLEM. WE AGREE TO DO THAT.

DR. JUELSGAARD: ALAN, I ACTUALLY ENVISION A LARGER DISCUSSION WHICH WOULD TALK ABOUT WHAT ARE THE POSSIBLE WAYS OF TRYING TO INTERACT WITH INDUSTRY? WHAT EXPERIENCES HAVE PEOPLE ON THE COMMITTEE HAD OR ARE AWARE OF? AND WHAT MIGHT OR MIGHT NOT WORK FOR US? SO IT WOULD BE A

PRESENTATION BY YOU GUYS, FINE, BUT ALSO A GRANDER DISCUSSION ABOUT HOW THINGS WORK OUT THERE.

DR. TROUNSON: I THINK THAT'S A GOOD IDEA. CURRENTLY THERE'S A BIT OF A CHANGE IN THE INDUSTRY BECAUSE SOME IPO'S MORE RECENTLY HAVE BEEN VERY SUCCESSFUL. BUT WE'VE HAD SOME INPUT THAT THAT'S PROBABLY NOT LONG LASTING, AND SO I THINK THIS DIALOGUE WOULD BE A GOOD THING BECAUSE WHEN COMPANIES ARE BEING WELL FUNDED, THEY HAVE A LOT LESS INTEREST, AND MAKES IT HARD TO WORK WITH THEM, OF COURSE, IF THEY'VE GOT MONEY FROM OTHER SOURCES. SO WE'D BE HAPPY TO DO THAT ON A BROADER SCALE AS WELL. ENDORSE THAT COMPLETELY.

CHAIRMAN THOMAS: SO, MR. SHEEHY, IS THAT OKAY, OR YOU'D STILL LIKE TO HAVE IT IN THE FORM OF A FRIENDLY AMENDMENT?

MR. SHEEHY: EITHER WAY. I TRUST MY COLLEAGUES.

MS. SAMUELSON: AS ONE ELEMENT I WOULD ASK THAT WE CONSIDER GETTING INFORMATION ABOUT THE -- OR THE CURRENT STATUS OR VIEW OF THE TWO TERMINATED CLINICAL TRIALS BECAUSE THEY ARE OUR MOST SIGNIFICANT ENDEAVORS. AND I DON'T THINK WE KNOW MUCH ABOUT WHY THEY DIDN'T SUCCEED.

CHAIRMAN THOMAS: WELL, I'M NOT SURE

THAT'S CORRECT. I THINK WE KNOW QUITE A BIT ABOUT WHAT HAPPENED ACTUALLY. BUT IN ANY EVENT --

MS. SAMUELSON: WE WELL MAY. I DON'T.

CHAIRMAN THOMAS: JEFF, IS IT OKAY JUST TO LEAVE IT AS IS? SO WE HAVE A MOTION ON THE TABLE. I THINK WE'VE PROBABLY BEATEN THIS ONE TO THE GROUND. CAN WE DO A VOICE VOTE ON THIS? ALL THOSE IN -- PLEASE RESTATE THE MOTION, MR. HARRISON.

MR. HARRISON: THE MOTION IS TO APPROVE THE CONCEPT PROPOSAL FOR A PROGRAM ANNOUNCEMENT FOR A CIRM INDUSTRY CO-FUNDING AGREEMENT.

CHAIRMAN THOMAS: DO WE HAVE ANY PUBLIC COMMENT BEFORE WE PROCEED TO A VOTE? HEARING NONE, ALL THOSE IN FAVOR PLEASE SAY AYE. OPPOSED? ON THE PHONE.

MR. GOLDBERG: AYE.

CHAIRMAN THOMAS: THANK YOU. MOTION CARRIES. OKAY.

IN THE FINEST TRADITION OF HAVING TO HAVE AT LEAST ONE ITEM ON THE AGENDA OUT OF ORDER, WE'RE GOING TO PROCEED TO THE COMMUNICATIONS UPDATE BECAUSE WE WANT TO GET TO KEVIN AND AMY DUE TO A LOGISTICAL ISSUE.

MR. MC CORMACK: CHAIRMAN THOMAS, MEMBERS OF THE BOARD, I'M GOING TO BE AD LIBBING WHILE AMY

IS FIXING THE TECHNICAL PROBLEM.

I WANTED TO BEGIN BY THANKING MY COLLEAGUE TODD DUBNIKOFF, WHO EDITED THE VIDEO, SHOT AND EDITED THE VIDEO THAT YOU SAW EARLIER TODAY, THE TRIBUTE TO DUANE. TODD PUT IN AN AWFUL AMOUNT OF WORK IN A VERY SHORT PERIOD OF TIME. HE SHOT SEVEN INTERVIEWS OVER THREE DAYS, INCLUDING TWO TRIPS TO SAN DIEGO AND A LONG DRIVE OUT TO PLEASANTON THAT SEEMED ALMOST AS FAR AWAY AS SAN DIEGO AT THE TIME, AND THEN HE ENDURED TWO DAYS WITH 102 DEGREE FEVER WHILE HE EDITED THE WHOLE PIECE TOGETHER. I THINK HE DID A REMARKABLE JOB, AND I JUST WANTED TO CONGRATULATE HIM AND TO THANK HIM FOR PERSEVERING WHAT I KNOW WAS KIND OF A DIFFICULT TIME FOR HIM.

TWO OTHER THINGS I WANTED TO MENTION, UPCOMING EVENTS. ONE IS PATIENT ADVOCATE MEETING. WE HAVE A PATIENT ADVOCATE MEETING IN LOS ANGELES ON SEPTEMBER 30TH. THIS IS THE SECOND OF OUR SERIES OF PATIENT ADVOCATE MEETINGS, AND THE FOLKS AT USC ARE GOING TO BE HOSTING US. SO WE'RE LOOKING FORWARD TO A GOOD MEETING THERE.

WE'RE ALSO HOLDING AN HIV COMMUNITY FORUM ON THE CONCEPT OF CURE. I'M WORKING WITH MR. SHEEHY ON ORGANIZING THAT. THAT'S GOING TO FEATURE PEOPLE FROM THE STEM CELL AGENCY, FROM CAL-IMMUNE, SAN

FRANCISCO GENERAL, UCSF, AND GLADSTONE INSTITUTE, AND A NUMBER OF OTHER RESEARCHERS AND COMMUNITY ACTIVISTS.

AND THE IDEA IS TO UPDATE THE COMMUNITY ON WHAT'S HAPPENING IN THE SITUATION WITH A CURE FOR HIV RATHER THAN JUST TALKING ABOUT TREATMENTS. IT'S A PRETTY EXCITING AREA, AND THERE'S A LOT TO TALK ABOUT. AND THE GOAL IS TO KIND OF CREATE A CONVERSATION, A DIALOGUE, THAT SHOWS EXACTLY WHERE WE'RE GOING, THE PROGRESS THAT'S BEING MADE, BUT ALSO THE CENTRAL ROLE THAT WE'RE PLAYING IN IT. SO I'LL BRING YOU AN UPDATE ON BOTH OF THOSE MEETINGS AT OUR BOARD MEETING IN OCTOBER.

ONE OF THE BIG THINGS WE'VE BEEN DOING OVER THE LAST FEW WEEKS WAS WITH THE CREATIVITY STUDENTS. ONE OF THE ELEMENTS WE ADDED TO THIS YEAR'S CURRICULUM WAS A SOCIAL MEDIA THING. WE ASKED ALL THE STUDENTS TO PROVIDE BLOGS, PHOTOGRAPHS, VIDEOS TO DOCUMENT THEIR EXPERIENCES IN WORKING WITH SOME OF THE BEST STEM CELL RESEARCHERS IN THE COUNTRY. AND WE GOT A HUGE RESPONSE. IT WAS REALLY GOOD. IT WAS FUN TO SEE THEM AND VERY GRATIFYING, BUT IT ALSO FED INTO ONE OF OUR OTHER GOALS, WHICH IS THE IDEA OF REACHING OUT TO NEW AUDIENCES AND TRYING TO USE SOCIAL MEDIA IN NEW WAYS

TO CREATE NEW AUDIENCES. AND MY COLLEAGUE AMY ADAMS, WHO HAD A BIG ROLE ALSO IN THE VIDEO THAT YOU SAW EARLIER, IS GOING TO TALK MORE ABOUT THAT NOW. THANK YOU.

MS. ADAMS: HI, MEMBERS OF THE BOARD, MEMBERS OF THE PUBLIC. THE LAST TIME I SPOKE TO YOU I WAS LETTING YOU KNOW THAT WE WERE LAUNCHING A NEW WEB SITE. THE WEB SITE HAS NOW BEEN LAUNCHED, AND I WANT TO TELL YOU A LITTLE BIT ABOUT SOME OF THE SUCCESS WE'VE HAD WITH THAT WEB SITE.

OVER THE PAST YEAR, SO LOOKING AT TRAFFIC TO THE WEB SITE THIS YEAR VERSUS LAST YEAR, TRAFFIC HAS GONE UP 47 PERCENT. WE'RE VERY HAPPY ABOUT THAT. TRAFFIC DUE TO SEARCH, SO PEOPLE SEARCHING FOR STEM CELL-RELATED TOPICS, THAT SEARCH HAS GONE UP BY ABOUT 30 PERCENT. AND THAT IS DUE TO SOME CHANGES THAT WE'VE MADE IN THE NEW WEB SITE. SO IT WAS ONE OF THE FEATURES OF THE NEW WEB SITE WE WERE LOOKING FORWARD TO IS THAT IT WOULD SEARCH BETTER.

AND THEN DIRECT TRAFFIC INCREASED 77 PERCENT. DIRECT TRAFFIC, WHAT THIS MEANS IS WE DON'T KNOW WHERE IT CAME FROM. SO IT'S PEOPLE WHO SENT A LINK TO SOMEONE ELSE VIA E-MAIL OR VIA TEXT MESSAGE, SOMEONE CLICKED ON THAT LINK AND GOT TO OUR WEB SITE. SO THE TAKE-AWAY HERE IS PEOPLE ARE

TALKING ABOUT US BECAUSE THEY ARE SENDING LINKS TO EACH OTHER, AND THEN PEOPLE ARE CLICKING ON THOSE LINKS. SO WE ALSO CONSIDER THAT TO BE GOOD NEWS.

AS KEVIN WAS SAYING, WE HAVE A VERY ACTIVE SOCIAL MEDIA PROGRAM, AND I WANT TO JUST GIVE YOU A GENERAL SENSE OF THE KINDS OF NUMBERS OF PEOPLE WE REACH THROUGH THESE PROGRAMS. SO I DON'T HAVE A POINTER. THAT'S C. THAT'S OUR ICON THAT REPRESENTS OUR WEB SITE. SO WE GET ABOUT -- IN THE LAST MONTH WE GOT 17,000 UNIQUE VISITORS TO OUR WEB SITE IN THE PAST MONTH. THAT'S LOW. IT'S USUALLY LIKE 20 TO 21,000 VISITORS TO OUR WEB SITE. I THINK PEOPLE ARE ON VACATION IN AUGUST. ALL OF OUR NUMBERS I'M SHOWING YOU ARE A LITTLE LOW.

THE NEXT ICON IS OUR YOUTUBE VIDEOS, MANY OF WHICH YOU HAVE SEEN AT BOARD MEETINGS. WE HAD MORE THAN 4,000 VIDEO VIEWS IN THE PAST MONTH. AND YOUTUBE TELLS US HOW LONG PEOPLE SPEND WATCHING VIDEOS. SO WE HAD 9.6 DAYS OF VIEW TIME. SO THAT'S LIKE TEN DAYS OUT OF 30 DAYS, ONE OUT OF EVERY THREE MINUTES PEOPLE ARE WATCHING VIDEOS ON OUR WEB SITE, ON YOUTUBE, AND OTHER PLACES.

SO THE NEXT ICON REPRESENTS OUR BLOG. THE BLOG HAD MORE THAN 5,000 VIEWS IN THE PAST MONTH. FACEBOOK, WE REACHED MORE THAN 62,000 PEOPLE. WE

HAVE E-MAIL LISTS. THE PRESS RELEASE LIST, LIKE THE ONE WE JUST USED TODAY TO SEND THE PRESS RELEASE. WE HAVE A MONTHLY NEWSLETTER, A VARIETY OF OTHER THINGS. 17,000 RECIPIENTS IN THE PAST MONTH. TWITTER WE REACHED 260,000 PEOPLE. AND THEN OUR LINKED-IN SITE HAS CLOSE TO 2,000 MEMBERS.

SO THESE ARE PRETTY BIG NUMBERS OF PEOPLE WE'RE REACHING WITH OUR CONTENT IN VARIOUS DEGREES OF DEPTH. OBVIOUSLY YOU DON'T GET A LOT OF MEAT OUT OF A TWITTER POST; BUT IF THAT TWITTER POST DRIVES YOU TO OUR BLOG OR TO OUR WEB SITE, WE CAN GIVE PEOPLE SOME PRETTY DETAILED CONTENT ABOUT THE KINDS OF INITIATIVES AND THE KINDS OF PROGRESS WE'RE MAKING.

AND THEN THIS BOTTOM ICON, THE TOP NUMBERS ARE JUST WHAT WE ARE DOING; BUT BECAUSE WE WORK VERY CLOSELY WITH THE COMMUNICATIONS PEOPLE AT PATIENT ADVOCACY GROUPS, AT THE GRANTEE INSTITUTIONS, THEIR BLOG ENTRIES, THEIR FACEBOOK ENTRIES, THEIR TWEETS, AND ALL THAT STUFF, I CAN TRACK THAT. IF THEY MENTION CIRM, I CAN TRACK IT. SO WE HAD CLOSE TO 500,000 PEOPLE REACHED THROUGH OTHER PEOPLE TALKING ABOUT US ON SOCIAL MEDIA. AND OBVIOUSLY THESE ARE NOT ALL UNIQUE PEOPLE, BUT SOME OF THEM ARE UNIQUE PEOPLE.

OKAY. THIS IS MEANT TO BE CONFUSING. BASICALLY JUST TO SHOW YOU THAT THERE IS QUITE AN INTERACTION BETWEEN THE DIFFERENT TOOLS. SO IF SOMEONE COMES TO OUR WEB SITE, WE CAN DRIVE THEM TO OUR SOCIAL MEDIA. WE LIVE IN A DAY AND AGE WHERE PEOPLE ARE NOT JUST SEARCHING THE WEB FOR INFORMATION. THEY ARE OFTEN RELAXING ON FACEBOOK. AND IF ON FACEBOOK ONE OF THEIR FRIENDS POSTS SOMETHING ABOUT CIRM, THEY CAN LEARN ABOUT US. THAT MIGHT DRIVE THEM TO GO TO OUR WEB SITE OR GO TO THE BLOG. SO WE'RE TRYING TO TRAP VIA WHATEVER IT IS THAT THEY ARE LOOKING AT, TWITTER, FACEBOOK, BLOG, AND DRIVE THEM TO MORE CONTENT ABOUT CIRM.

AND FINALLY, I WANTED TO SORT OF SHOW YOU HOW THE DIFFERENT SOCIAL MEDIA EFFORTS WORK TOGETHER. WE'RE NOT JUST POSTING ONE THING ON THE BLOG AND SOMETHING ELSE ON TWITTER. WE TRY TO GET SOME SYNERGY OUT OF THE DIFFERENT TOOLS. AND A GREAT EXAMPLE OF THIS IS KEVIN JUST MENTIONED THAT WE HAD A SOCIAL MEDIA PROGRAM WITH THE CREATIVITY STUDENTS. SO THEY SENT US VIDEOS, THEY SENT US IMAGES, THEY SENT IS BLOG ENTRIES. WE POST THE VIDEOS ON YOUTUBE AND THE WEB SITE. WE POST INSTAGRAM PHOTOS ON THE WEB SITE, WE TWEET, AND WE FACEBOOK ABOUT ALL THE IMAGES IN THE VIDEOS. WE

POSTED THE BLOGS. THE BLOGS ARE FABULOUS. IF PEOPLE -- I THINK SOME OF YOU HAVE SEEN SOME OF THESE BLOG ENTRIES, AND YOU GET A REAL INSIGHT INTO WHAT THE KIDS LEARNED OVER THE SUMMER. IT'S PRETTY FUN READING.

ALL THIS TOGETHER HAS ADDED UP TO, WITH THE CREATIVITY AWARDS, MORE THAN 150,000 INDIVIDUAL TIMES WHEN PEOPLE HAVE SEEN CONTENT ABOUT OUR CREATIVITY STUDENTS THROUGH ONE OF THESE MECHANISMS.

AND THEN IN THE PAST WE'VE TALKED TO YOU ABOUT THE ELEVATOR PITCH CHALLENGE. AND THAT PROGRAM REACHED MORE THAN 250,000 PEOPLE. AND THAT WAS BOTH THROUGH OUR WORK, BUT ALSO BECAUSE WE WORKED WITH OUR GRANTEE INSTITUTIONS. THEY WERE BLOGGING AND TWEETING, FACEBOOKING, AND ALL THAT. SO THOSE 150,000 AND 250,000 NUMBERS ALSO ENCOMPASS THE EFFORTS OF OUR GRANTEES AND OTHER PEOPLE.

SO THAT'S THE UPDATE I WANTED TO GIVE YOU. ARE THERE ANY QUESTIONS? EXCELLENT.

CHAIRMAN THOMAS: THANK YOU, KEVIN, AMY, AND TODD, AND DON AS WELL, FOR ALL YOUR HARD WORK ON THE COMMUNICATIONS.

I SHOULD NOTE JUST A SIDEBAR COMMENT ON THE CREATIVITY AWARDS. FOR THOSE THAT WERE ABLE TO GO, IT WAS A TYPICALLY FANTASTIC EVENT WHERE THESE

HIGH SCHOOL KIDS ARE PRESENTING ON WORK THAT THEY'VE DONE THROUGH CIRM'S ABILITY TO FUND THEIR RESEARCH. AND WHILE THE PRESENTATIONS TENDED TO BE MIXED IN TERMS OF THE KIDS THEMSELVES, AT A MINIMUM THEY WERE ALL REMARKABLE FOR THEIR HIGH DEGREE OF ENTHUSIASM AND THE SCOPE OF WORK. AT THE TOP, WHEN YOU HIT SOME OF THESE KIDS, YOU COULDN'T BELIEVE YOU WERE HEARING FROM SOMEBODY WHO IS ONLY INVOLVED FOR EIGHT WEEKS OR SO. AND THEY CAME TO HAVE SUCH AN INCREDIBLE GRASP IN SUCH A SHORT PERIOD OF TIME I THINK IS A TESTIMONY TO THE PROGRAM.

SO I RECOMMEND THESE EVENTS TO ANY OF YOU WHO'VE NOT SEEN THEM. THEY'RE GREAT. ONE SUCH EVENT AS WELL WAS AT USC WHICH SENATOR TORRES, MARIA, AND I AND KEVIN ATTENDED WHERE THEY TALKED TO A NUMBER OF THE KIDS WHO WERE INVOLVED THERE FROM VARIOUS HIGH SCHOOLS IN LOS ANGELES AND INCLUDING A MOST FASCINATING PANEL DISCUSSION WHERE THESE KIDS WERE ASKED QUESTIONS ABOUT THEIR THOUGHTS ON THE PROGRAM AND GAVE WONDERFULLY CANDID COMMENTARY AND HIGHLY INFORMED, EVEN THOUGH AT THAT POINT THOSE KIDS HAD ONLY BEEN IN THE PROGRAM FOR TWO WEEKS. SO IT SORT OF GOES TO SHOW THAT WE'VE GOT A TERRIFIC PIPELINE IN THE MAKING THROUGH THESE KIDS AND OTHERS THAT HAVE GONE THROUGH THE PROGRAM.

MR. HARRISON NOTED THAT I'D BE REMISS IN SAYING THAT WE TOOK NO ACTION IN OUR CLOSED SESSION TODAY JUST FOR THE RECORD.

WE'LL PROCEED NOW TO THE ALWAYS MOST CONTROVERSIAL TOPIC, WHICH HERE IS ITEM 13, APPROVAL OF LAST WEEK'S BOARD MINUTES. DO I HEAR A MOTION. SO CONTROVERSIAL, NOBODY EVEN MOVES.

MR. HARRISON: WE'VE LOST OUR QUORUM.

CHAIRMAN THOMAS: OKAY. NEXT TIME. FORTUNATELY WE HAVE NO REAL ITEMS TO APPROVE HERE LEFT. LAST THING ON THE AGENDA BEFORE WE GET TO THE ULTIMATE, WHICH WOULD BE THE PRESIDENT'S REPORT, WOULD BE A REPORT ON THE CONTRACTS. MR. STEIN.

DR. STEIN: GOOD AFTERNOON. EACH YEAR THE CONTRACTING POLICY CALLS ON STAFF TO MAKE A REPORT TO THE BOARD LISTING ALL OF CIRM'S CONTRACTS FOR MORE THAN $20,000. AND THE REPORTING PERIOD FOR THIS UPDATE IS FISCAL YEAR '12-'13. SO THAT'S JULY 2012 THROUGH JUNE 30, 2013. AND THE REPORT IS IN YOUR MATERIALS. AND STAFF IS HERE TO ANSWER ANY QUESTIONS YOU MAY HAVE. IF THERE ARE NO QUESTIONS, THAT'S THE UPDATE.

CHAIRMAN THOMAS: MR. JUELSGAARD.

DR. JUELSGAARD: CAN'T GET AWAY WITHOUT BEING ASKED A QUESTION. LET ME ASK YOU JUST IN

DETERMINING -- YOU HAVE A COLUMN, THE SECOND COLUMN OVER BEYOND PURPOSE, AMOUNT AVAILABLE FOR FY '12-'13. SO WHEN IS THAT AMOUNT ESTABLISHED? IS THAT ESTABLISHED AT THE TIME THE BUDGET IS PREPARED? THE AMOUNT THAT'S AVAILABLE, YOU LINE ITEM BUDGET LIKE THIS OR WHAT?

DR. STEIN: THAT'S MY UNDERSTANDING OF THE PROCESS. YES, WHEN THE BUDGET IS ESTABLISHED. ANY OTHER QUESTIONS?

CHAIRMAN THOMAS: VERY EFFICIENTLY DONE, MR. STEIN. CONGRATULATIONS. OKAY.

SO I THINK NOW WE ARE TO DR. TROUNSON ON THE ASSUMPTION THAT HIS TOOTH WILL PERMIT HIM TO SPEAK, HAVING HAD A BIT OF A DENTAL EMERGENCY MIDMEETING.

DR. TROUNSON: THANK YOU, CHAIR. I'M SORRY ABOUT THAT. IN THE MIDDLE OF THE MEETING, I HAD TO GO AND GET SOME SURGERY BECAUSE I'M LEAVING TONIGHT FOR MOROCCO, SO YOU SHOULDN'T FEEL BADLY FOR ME, BUT I HAVEN'T SEEN MY WIFE FOR SEVEN MONTHS. SO I DIDN'T WANT A TOOTHACHE IN THE DESERT. SO I WENT TO VISIT THE DENTIST AND A VERY GOOD DENTIST IN SAN DIEGO. THANK YOU VERY MUCH, DENTIST.

SO THE PRESIDENT'S REPORT IS A LITTLE LATE, BUT THAT'S THE WAY IT IS. I SET THIS UP

BEFORE WE HAD THE PRESENTATION THIS MORNING. AND I, LIKE ALL OF YOU, I THINK I JUST MISS MY FRIEND. AND I HAD A COUPLE OF SLIDES IN HERE BECAUSE I THOUGHT STARTING THE PRESIDENT'S REPORT WITH SOMETHING TO DO WITH DUANE ON THIS OCCASION WOULD REALLY BE INAPPROPRIATE. AND HE REALLY DID HAVE AN INCREDIBLE IMPACT, AND I HAD A LOT OF SLIDES WHICH I LEFT AWAY, BUT I JUST LEFT A FEW REALLY IMPORTANT ONES BECAUSE CONNECT HAS REALLY BEEN A VERY IMPORTANT ORGANIZATION. AND IT'S HELPED MORE THAN 2,000 COMPANIES SINCE 1985 AND IS WIDELY REGARDED AS THE WORLD'S MOST SUCCESSFUL REGIONAL PROGRAM LINKING INVENTORS AND ENTREPRENEURS WITH THE RESOURCES AND NEED FOR COMMERCIALIZATION OF PRODUCTS.

I'M NOT SURE THAT EVERYBODY KNOWS THAT. I WASN'T AWARE OF HOW MUCH THEY'D REALLY DONE. SO THIS PROGRAM HAS BEEN MODELED IN ALMOST 40 REGIONS IN THE WORLD. SO THIS IS A REALLY IMPORTANT PROGRAM THAT DUANE WAS RUNNING. AND THE KEY TO THEIR SUCCESS HAS BEEN THE UNIQUE CONVEYER BETWEEN THE INDUSTRY, CAPITAL SOURCES, PROFESSIONAL SERVICE PROVIDERS, AND RESEARCH ORGANIZATIONS, WHICH IS RIGHT IN OUR SWEET SPOT WHERE WE'RE TRYING TO OPERATE. SO THAT'S WHY IT WAS -- HE WAS REALLY SO IMPORTANT FOR US AND ALSO FOR THE REGION.

AND I THINK THIS SAN DIEGO IS A VERY SUCCESSFUL LIFE SCIENCE CLUSTER BECAUSE OF CONNECT AND BECAUSE OF THE WAY THAT HE WAS ABLE TO MAKE THESE THINGS HAPPEN HERE IN A REALLY, I THINK, REALLY EXTRAORDINARY WAY. AND IF YOU LOOK THROUGH THE CLUSTER REPORT ON SAN DIEGO, THE INFORMAL CONTACT AND PARTICIPATION OF PROFESSIONAL NETWORKS ARE VERY, VERY HIGH HERE. AND THEY'RE THE LEADING TYPES OF COLLABORATIONS IN THE CLUSTER FOLLOWED BY COOPERATION IN EDUCATION, CONTRACT RESEARCH, AND ADVISORY. THESE ARE THE SORT OF THINGS THAT THESE PEOPLE ACTUALLY WORK TOGETHER. AND YOU SEE IT MORE HERE IN SAN DIEGO THAN I THINK I'VE EVER SEEN REALLY ANYWHERE.

THE OTHER POPULAR TYPES OF COLLABORATIONS INCLUDE THE MOBILITY OF PEOPLE, PEOPLE MOVING FROM ACADEMIA TO INDUSTRY, INDUSTRY BACK INTO ACADEMIA. THE MOVEMENTS AROUND THERE HAVE BEEN REALLY HIGH AT 40 PERCENT, COOPERATION IN R&D 40 PERCENT, AROUND SHARING OF FACILITIES 40 PERCENT, AND PUBLICATIONS ARE OVER 30 PERCENT. SO THESE ARE PEOPLE WORKING TOGETHER MAKING A VERY, VERY SUCCESSFUL CLUSTER. AND HE WAS REALLY QUITE IMPORTANT IN ALL OF THAT, AND CONNECT WAS REALLY THE EMBODIMENT OF HOW THIS ALL SORT OF WORKED AND SO WAS DUANE.

SO THAT'S WHY, FOR MANY REASONS, WE'LL MISS HIM BECAUSE IT'S JUST A NATURAL WAY OF GETTING PEOPLE TO WORK TOGETHER. SINCE I'VE BEEN HERE, MY EFFORTS HAVE REALLY BEEN TRYING TO GET THE BEST PEOPLE WORKING TOGETHER. AND THAT'S WHAT I ALWAYS HAVE BEEN TRYING TO DO, AND I THINK WE'VE BEEN REASONABLY SUCCESSFUL, BUT DUANE WAS ALWAYS SAYING YOU JUST HAVEN'T GOT TO THE TOP OF IT YET. KEEP GOING BECAUSE IT IS IMPORTANT TO LINK THE BEST WITH THE BEST, AND WE'VE GOT THE BEST STEM CELL RESEARCH IN THE WORLD. WE'VE GOT SOME OF THE BEST BIOTECH COMPANIES HERE IN CALIFORNIA. WE'VE GOT A GROWTH INDUSTRY GOING HERE. THINGS ARE LOOKING VERY POSITIVE. KEEP GOING BECAUSE THIS IS GOING TO BE A VERY SUCCESSFUL OUTCOME.

AND SO IN ME STILL BEING HERE AT CIRM SAYS A LOT ABOUT WANTING TO DO THE KIND OF THINGS THAT DUANE HAD STRONGLY IN HIS MIND.

NOW, I'M GOING TO TELL YOU ABOUT A COUPLE OF RESEARCH REPORTS BECAUSE I THINK THAT'S WHAT YOU REALLY SHOULD ALWAYS GET FROM ME. AND THIS FIRST ONE IS REALLY ABOUT REPOPULATION OF DECELLULARIZED HEART TISSUE WITH HUMAN PLURIPOTENTIAL STEM CELLS. CAN YOU BUILD AN ARTIFICIAL HEART? CAN YOU REALLY DO THAT? I THINK A YEAR OR TWO YEARS AGO WE WOULD

HAVE SAID, WELL, IT'S A GREAT THOUGHT, BUT YOU'D NEVER DO IT. WELL, THIS GROUP HERE AT THE UNIVERSITY OF PITTSBURGH, IN FACT, HAVE BEEN ABLE TO DO IT TO A POINT.

SO THEY'VE USED HUMAN IPS CELLS DIFFERENTIATED INTO MULTIPOTENTIAL CARDIAC PROGENITOR CELLS WHICH FORM CARDIOMYOCYTES, THE HEART MUSCLE CELLS, AND SMOOTH MUSCLE, AND ENDOTHELIAL CELL TYPE. SO THEY FORM ALL OF THOSE THREE TYPES. AND THEY POPULATED DECELLULARIZED MOUSE HEARTS. I'LL SHOW YOU HOW THEY DID THAT IN A MOMENT. SO THEY TAKE ALL THE CELLS OUT OF A MOUSE HEART AND THEN THEY REPOPULATE IT WITH THESE HUMAN CELLS AND THEN GET THE THING TO WORK TOGETHER. AND IN THE LABORATORY THEY USED 20 DAYS OF PERFUSION TO SORT OF GET THESE CELLS FUNCTIONAL. THEN THEY LOOKED AT WHETHER THESE CELLS WORKED TOGETHER TO FORM A HEART. AND THEY SHOWED SPONTANEOUS CONTRACTIONS.

SO IF YOU GO AND LOOK UP THIS PAPER IN *NATURE COMMUNICATIONS*, YOU WILL SEE THIS HEART, THIS HEART THAT DIDN'T HAVE A SINGLE CELL IN IT BEATING. AND YOU WILL ALSO SEE THAT IT'S GOT A MECHANICAL FORCE. THIS ACTUALLY CAN FORCE -- WILL BE ABLE TO FORCE BLOOD OR COULD FORCE FLUID THROUGH IT, AND

IT'S ALSO RESPONSIVE TO DRUGS.

SO WHAT THEY DID WAS TO MAKE IPS CELLS FROM AN ADULT AND DRIVE THOSE CELLS INTO THE MULTIPOTENTIAL CELLS. AND THEN ON THE RIGHT-HAND SIDE YOU CAN SEE THE COMPLETELY CELLULARIZED HEART RIGHT UP ON THE TOP LEFT IN THE PHOTOGRAPHS OF THE HEART THERE ATTACHED UNDER NO. 1 THERE, IF YOU CAN READ THAT. AND AS YOU MOVE THROUGH IT, YOU CAN SEE THAT THE CELLS HAVE BEEN PROGRESSIVELY TAKEN OUT. SO THEY USE A DETERGENT TO REMOVE THE CELLS AND LEAVE THE EXTRACELLULAR MATRIX OF THE HEART INTACT. SO BY THE TIME YOU GET TO SEVEN, YOU CAN SEE THAT THERE'S REALLY NO CELLS IN THAT HEART, AND THE BLUE STAINING JUST LEAVES SOME OF THE MATRIX THERE.

YOU LOOK DOWN BELOW THERE'S A MEASUREMENT OF DNA. SO AN INTACT MOUSE HEART HAS A WHOLE RED BAR FULL OF DNA AND THE DECELLULARIZED HEART HAS NONE. SO ALL THE CELLS HAVE GONE. WHAT THEY DO NEXT IS THEN THEY GROW THE IPS CELLS FOR SIX DAYS IN CULTURE, SUBJECT THEM TO EXPOSURE TO DIFFERENT GROWTH FACTORS OVER THAT PERIOD OF TIME, AND THEN AT DAY SIX THEY SEPARATED ALL THE CELLS, AND THEN THEY PUT THOSE CELLS INTO THE SOLUTION WITH THE DECELLULARIZED HEART. AFTER ANOTHER 20 DAYS, THAT'S A TOTAL OF 26 DAYS, THEY FOUND CARDIOMYOCYTES,

SMOOTH MUSCLE, AND ENDOTHELIAL CELLS. YOU CAN SEE THERE'S AN EKG THERE THAT SHOWS YOU THAT THERE'S AN EKG ACTUALLY FUNCTIONING IN THESE CELLS. AND THERE'S INTRACELLULAR CALCIUM TRANSIENTS SHOWN DOWN ON THE BOTTOM THERE.

SO HERE IS THE FIRST RUDIMENTARY HEART THAT I'VE SEEN IN THE LABORATORY THAT'S ACTUALLY BEEN DEVELOPED WITH HUMAN IPS OR PLURIPOTENTIAL STEM CELLS. NOW, THE IMPORTANT PART OF THIS IS THAT YOU'RE ABLE TO DO THAT IN A SMALL HEART LIKE A MOUSE, DECELLULARIZED MOUSE, WITH HUMAN CELLS. IT WOULDN'T BE MUCH USE TO THE MOUSE BECAUSE THE HEART RATE IS MUCH HIGHER IN A MOUSE THAN IT IS IN A HUMAN. IT BEATS VERY MUCH FASTER. NEVERTHELESS, THIS IS THE BEGINNINGS TOWARDS MAKING A HEART.

AND I WILL COME BACK TO YOU AT SOME OTHER TIME BECAUSE WE'VE ENGAGED THE WHITE HOUSE IN THIS AREA OF LOOKING TO A GRAND CHALLENGE FOR WHICH I AND MY COLLEAGUES HERE AT CIRM HAVE PUT A PROPOSAL TO THE WHITE HOUSE TO RUN A GRAND CHALLENGE ON CURE OF HEART DISEASE. SO WE'LL COME BACK TO YOU. THE WHITE HOUSE, ALL I CAN SAY AT THE, MOMENT IS VERY INTERESTED, AND WE'LL SEE IF WE CAN GET THEM TO ENGAGE WITH US AND WITH OTHER PEOPLE TO DO, I THINK, A SPECTACULAR PROJECT. SO THIS IS IN EARLY DAYS,

BUT HERE IS GOOD SORT OF INDICATION THAT YOU CAN DO SOME OF THAT KIND OF THING.

THE ONLY OTHER PUBLICATION I WANT TO DRAW YOUR ATTENTION TO WAS WORK FROM MICHELLE SADELAIN'S LAB AT THE SLOAN KETTERING IN NEW YORK WHERE THEY HAVE USED COMBINED INDUCED PLURIPOTENTIAL STEM CELL TECHNOLOGY AND COMBINED WITH THE CAR TECHNOLOGY CALLED THE CHIMERIC ANTIGEN RECEPTOR. THIS IS SOMETHING I THINK JEFF WOULD KNOW ABOUT, DOES KNOW ABOUT. I KNOW THAT.

SO THEY'VE GENERATED HUMAN T-CELLS TARGETED TO CD 19. IT'S AN ANTIGEN EXPRESSED BY MALIGNANT B CELLS IN TISSUE CULTURE. THESE IPS CELL-DERIVED CAR EXPRESSING T-CELLS, THEY DISPLAY A PHENOTYPE RESEMBLING THAT OF INNATE GAMMA DELTA T-CELLS. THESE ARE VERY SIMILAR TO CAR-TRANSDUCED PERIPHERAL BLOOD GAMMA DELTA T-CELLS. AND THESE IPS-DERIVED T-CELLS POTENTLY INHIBIT TUMOR GROWTH IN A XENOGRAPH MODEL. SO THEY'RE VERY, VERY EFFECTIVE.

SO THIS IS THE FIRST ONE THAT I'VE REALLY SEEN WHERE THEY'VE GENERATED THESE TUMOR TARGETING LYMPHOCYTES THAT GO AFTER A CANCER IN A VERY, VERY EFFECTIVE WAY. AND YOU CAN DEVELOP THOSE USING AN IPS CELL-TYPE STRATEGY, WHICH WOULD MEAN THAT THE T-CELLS THAT ARE FORMED SHOULD NOT BE REJECTED.

THESE ARE AN IMMUNE CELL, AND YOU CAN GIVE THEM THIS DOUBLE BAIT, IF YOU LIKE, OF A T-CELL RECEPTOR AND THE CAR, AND THEY'LL GO AFTER, VERY VICIOUSLY GO AFTER THOSE CANCER CELLS EXPRESSING THAT.

WE'VE ALREADY GOT SEVERAL PROJECTS IN THE PORTFOLIO THAT ARE USING THIS KIND OF APPROACH, BUT HAVE NOT DEVELOPED THEM FROM IPS CELLS. I THINK IT'S A VERY, VERY NICE PIECE OF WORK. I LIKE THE CAR APPROACH. I THINK IT'S GOING TO BE VERY EFFECTIVE. I THINK THE PEOPLE AT UCLA AND THE OTHERS WHO WE ARE SUPPORTING WILL REALLY DEMONSTRATE THIS RATHER NICELY, I THINK. AND I SEE THIS AS A REALLY BIG STEP FORWARD IN AN ATTACK ON CANCERS. SO I THOUGHT IT WAS A VERY NICE PIECE OF WORK.

SO NOW GOING THROUGH THE CURRENT RFA PROGRAM, AS I USUALLY DO FOR YOU, WE'VE DONE THE TRANSLATION MEETING. THE DISEASE TEAM III, WHICH IS THE BIG PROGRAM, THAT'S IN SEPTEMBER. THE GRANTS REVIEW WILL BE IN SEPTEMBER. SO WE'RE ALL LOOKING FOR THAT. THAT'S THE THIRD DISEASE TEAM PROGRAM WE'VE GOT. IT ALWAYS INDUCES LOTS OF ENERGY AND LOTS OF SECRETIONS, THIS WORK, I TELL YOU. AND SO ONE WAY OR ANOTHER WE'LL GET EXCITED. THAT'S FOR CERTAIN.

BASIC BIOLOGY V, SO THOSE REVIEW

APPLICATIONS ARE IN OCTOBER. AND GENOMICS, WE'RE COMING BACK WITH GENOMICS, A REVIEW OF THE APPLICATIONS IN NOVEMBER. AND I'M FEELING VERY CONFIDENT ABOUT THAT REVIEW NOW. SO WE'VE GOT A TERRIFIC TEAM. IT'S SOME OF THE ORIGINAL PEOPLE, BUT WE'VE GOT A GREAT GROUP OF GENOMICS PEOPLE COMING. AND I THINK WE'VE GOT ENHANCED APPLICATIONS THAT WILL GIVE US WHAT WE REALLY WANT NOW.

STRATEGIC PARTNERSHIP III, THAT WILL BE POSTED -- THAT HAS BEEN POSTED IN JULY. ALREADY BEEN POSTED.

RESEARCH LEADERSHIP EXTENSIONS, THE AMENDED RFA WILL POST SOMETIME IN AUGUST. I DON'T THINK IT'S BEEN POSTED YET.

TOOLS AND TECHNOLOGIES III, POSTING IN SEPTEMBER.

AND OUR ALPHA CLINICS WE EXPECT TO POST IN OCTOBER, NOVEMBER. SO THERE'S PLENTY OF WORK TOWARDS THE END OF THIS YEAR FOR US ALL, PLENTY OF GRANTS REVIEWS AND PLENTY OF WORK FOR THE BOARD COMING UP.

WE HAD THE SCIENTIFIC ADVISORY BOARD MEETING. YOU REMEMBER THE INSTITUTE OF MEDICINE RECOMMENDED THAT WE HAVE A SCIENTIFIC ADVISORY BOARD. SO THE ADVISORY BOARD, SEVEN OF THE EIGHT

ADVISORY BOARD MEMBERS ATTENDED IN SAN FRANCISCO AT CIRM. SIR JOHN BELL FROM OXFORD WAS THE CHAIR. CHRISTINE MUMMERY, SEAN MORRISON, STU ORKIN, FIONA WATT, JOHN WAGNER, AND COREY GOODMAN WERE THERE. THESE ARE A FEROCIOUS GROUP OF PEOPLE, I TELL YOU. THEY ARE VERY, VERY INTERESTED AND VERY STRONG VIEWS, OF WHICH THEY GAVE ME AN INSIGHT INTO WHAT THEIR REPORT WOULD BE.

I THINK IT'S VERY INTERESTING. THEY'LL COME BACK IN OCTOBER. I MIGHT BE HERE, BUT ELLEN WILL BRING IT FORWARD FOR ME BECAUSE I'LL BE IN EUROPE. I'M AT A NATURE MEDICINE MEETING LOOKING AT REGENERATIVE MEDICINE WORLDWIDE, AND I THOUGHT I NEEDED TO BE THERE. BUT THE SAB WILL BE PRESENTED TO YOU WITH OUR COMMENTS, MANAGEMENT'S COMMENTS, IN OCTOBER. AND I THINK YOU ARE GOING TO BE VERY INTERESTED IN THAT. I THINK YOU REALLY WILL.

SO THEY EXAMINED THE PROGRESS, POSITION, AND FUTURE PROSPECTS OF CIRM WITH THE FUNDING THAT'S AVAILABLE, AND THEY ARE PROVIDING RECOMMENDATIONS ON THE FUTURE FOCUS OF CIRM TO MAXIMIZE THE BENEFITS AND RECOGNITION OF CIRM. SO IN A DOUBLE ANGLE FOR US. AND EXPECT THOSE RECOMMENDATIONS AND MANAGEMENT'S COMMENTS IN THE OCTOBER MEETING.

UPCOMING MEETING, THERE'S A CIRM

SYMPOSIUM: BREAKING THE BOTTLENECK. YOU MAY NOT KNOW, BUT WE'RE HAVING SOME PROBLEMS IN GETTING HEMATOPOIETIC STEM CELLS, THE BLOOD CELL THAT GOES TO FORM ALL THE BLOOD IN THE BODY, WE ACTUALLY CAN'T GET THAT CELL TO COME FROM AN EMBRYONIC OR AN INDUCED PLURIPOTENTIAL CELL THROUGH TO A HEMATOPOIETIC STEM CELL THAT CAN ENGRAFT IN THE BONE MARROW. WE CAN MAKE WHAT APPEARS TO BE YOKE-TYPE HEMATOPOIETIC STEM CELLS. SO WE'RE ASKING AT THIS WORKSHOP TO SEE WHAT THE BOTTLENECK IS. I HAVE A FEW VIEWS ON IT, AND I WON'T ACTUALLY BE AT THE WORKSHOP, BUT I'VE TRANSMITTED IT TO THE MEMBERS. AND WE'LL GIVE YOU A READOUT OF WHAT THE EXPERTS THINK ON WHAT WE SHOULD DO TO GET THAT DONE. THIS IS HANDICAPPING US IN GETTING SOME WORK DONE WITH THE BLOOD DISEASES AND CANCERS NOT BEING ABLE TO DO THAT.

THERE'S A WORKSHOP ON OPTIMIZING EXPECTATIONS, SUCCESS, AND FIRST-IN-HUMAN TRIAL WITH STEM CELL THERAPIES IN SEPTEMBER. AND CIRM IS ON THE PROGRAM COMMITTEE. SO ELLEN WILL TELL YOU SOMETHING ABOUT THAT IN DUE COURSE. I THINK IT'S ELLEN AND ELONA AT THAT, IS IT?

THERE'S A CIRM-SPONSORED INTERNATIONAL WORKSHOP ON REGULATORY PATHWAYS. STEM CELL-BASED

THERAPIES ALSO IN SEPTEMBER AT BETHESDA AT THE NIH. STEM CELL AWARENESS DAY IS ON OCTOBER 2D WHERE WE TRY AND MAKE PEOPLE AWARE OF STEM CELL AND STEM CELL RESEARCH. IT'S A VERY GOOD PROGRAM, AND IT INVOLVES A LOT OF US AND THE SCIENTISTS AROUND CALIFORNIA, INDEED AROUND THE WORLD, TALKING TO STUDENTS, TALKING TO PEOPLE WHO ARE INTERESTED, GOING TO SCHOOLS, GOING TO PLACES TO INFORM.

CIRM-SPONSORED WEBINAR ON PARKINSON'S DISEASE: MOVING STEM CELL-BASED THERAPIES TO THE CLINIC IS ON NOVEMBER 14.

THE CIRM WORKS WITH THE FDA ON REGULATORY PATHWAYS FOR CELL THERAPY. AND AS I'VE STEPPED BACK OUT OF THAT, ELLEN HAS STEPPED FORWARD. SO THERE'S A CIRM WEBINAR ON MOVING CELL-BASED THERAPIES TO THE CLINIC FOR PARKINSON'S DISEASE. SO THAT'S, AS I JUST SAID, NOVEMBER 14. THERE'S ALSO THE REGULATORY PATHWAYS ON SEPTEMBER 17TH. I ALREADY SAID THAT AS WELL.

MEETINGS THAT HAVE BEEN HELD. J.T. JUST SPOKE TO YOU BEFORE THIS ON THE CREATIVITY POSTER DAY. A FEW YEARS AGO WE SET UP THE CREATIVITY AWARDS FOR STUDENTS, HIGH SCHOOL STUDENTS, TO EXPOSE THEM TO STEM CELLS AND SOMETHING ELSE. CREATIVITY COMES FROM LOOKING AT SEVERAL THINGS USUALLY AT

ONCE. SO IT'S THE INTERSECTION OF DISCIPLINES THAT CREATES A LOT OF THE CREATIVITY. AND THIS PROGRAM HAS BEEN, I THINK, FANTASTICALLY SUCCESSFUL. AND I THINK EVERYBODY WHO'S EXPOSED TO THOSE YOUNG PEOPLE ARE INCREDIBLY IMPRESSED. AND THEY GIVE UP THEIR SUMMERS TO BECOME INTERNS IN THE PROGRAMS. AND THEY'RE NOT JUST WASHING UP AND HOLDING ONTO THINGS. THEY'RE ACTUALLY RIGHT IN THERE DOING THE WORK. WHEN YOU GET TO MEET THEM, YOU ALMOST THINK THEY'RE PH.D. STUDENTS. THEY'VE REALLY GOT THE FIRE.

NIH REGENERATIVE MEDICINE INTERACTIONS WITH INDUSTRY WAS ON AUGUST 21ST. I THINK THAT WAS WHERE ELLEN WAS. AND IF YOU CARE TO ASK HER ABOUT THAT, THAT WOULD BE GOOD IF YOU WANTED TO.

CREATIVITY AWARDS DAY, THERE WERE ALL NINE FUNDED INSTITUTIONS THERE, 70 INTERNS PRESENTING POSTERS. NINE OF THOSE STUDENTS PRESENTED SHORT TALKS, AND I UNDERSTOOD THEY WERE VERY, VERY GOOD FROM THE SCIENCE STAFF THAT WERE THERE. AND THERE WERE 210 ATTENDEES INCLUDING 65 PARENTS AND FAMILY MEMBERS, BOARD MEMBERS, AND CIRM STAFF. SO IT WAS A REAL KIND OF CELEBRATION OF THESE YOUNG PEOPLE. AND I TELL YOU WHAT. THEY'RE KEYED INTO GOING INTO THE SCIENCE AND MEDICINE, THESE YOUNG PEOPLE NOW, AND THEY WILL BE CONTRIBUTORS IN SCIENCE AND MEDICINE IN

THE FUTURE.

INDUSTRY ENGAGEMENT AND SUPPORT, WHICH IS REALLY THE PROGRAM THAT ELONA RUNS, THERE WAS A REGENERATIVE MEDICINE VC MEET-UP THAT WAS HELD IN JUNE WHERE THEY HAD A SURVEY OF THREE OF THE 15 VC'S. THE PROGRAM WAS VERY WELL RECEIVED. AT LEAST ONE FOLLOW-UP DISCUSSION IS PLANNED WITH COMPANIES, AND THE PANELS INCLUDED GLOBAL IMPACTS, REIMBURSEMENT, FINANCING, OVERCOMING REGULATORY HURDLES TO REGENERATIVE MEDICINE. AND SIX CIRM GRANTEES PARTICIPATED IN THE PRESENTATION SESSIONS.

SO I THINK WHAT WE'RE TALKING ABOUT HERE IS THE MEETING ON THE MESA. SO THE SPEAKERS INCLUDE CELIA WITTEN FROM FDA. THAT'S OFTEN DIFFICULT TO GET CELIA TO COME VISIT, SO IT'S GREAT TO HAVE HER THERE. PETE SCHULTZ WILL ALSO BE SPEAKING. HE'S THE FOUNDING DIRECTOR OF THE SCRIPPS RESEARCH -- NO. HE'S THE FOUNDING DIRECTOR OF THE CALIFORNIA INSTITUTE FOR BIOMEDICAL RESEARCH, A NEW INSTITUTE DOWN THERE.

GRANTS MANAGEMENT SYSTEMS, I WON'T GO INTO THIS, BUT THE IP WORK AT THE INSTITUTE GOES ON. IT'S FANTASTIC. IT'S DOING REALLY WELL. IT REALLY HELPS US TO GET ALL OUR WORK DONE EFFECTIVELY AND EFFICIENTLY. AND THE TEAM REALLY ARE DOING A

FANTASTIC JOB.

I'LL FINISH AND GET CHILA TO DO THE FINANCIAL REPORT FOR YOU.

MS. SILVA-MARTIN: THANK YOU, DR. TROUNSON. GOOD AFTERNOON, MR. CHAIR, MEMBERS OF THE BOARD, MEMBERS OF THE PUBLIC. I KNOW IT'S REALLY LATE, SO I WILL TRY TO KEEP MY FINANCE REPORT SHORT AND BRIEF. TODAY I'M GOING TO REPORT ON THE FINAL OPERATING EXPENDITURES FOR THE '12-'13 FISCAL YEAR AS WELL AS GIVE YOU A BRIEF HIGHLIGHT ON OUR FINANCES FOR THE '13-'14 FISCAL YEAR AND BEYOND.

SO THIS FIRST SLIDE PROVIDES YOU WITH A HIGH LEVEL OVERVIEW OF OUR '12-'13 OPERATING EXPENDITURES AND GRANT DISBURSEMENTS FOR THE '12-'13 FISCAL YEAR AS COMPARED TO THE PRIOR PERIOD OF '11-'12. I'M NOT GOING TO GO INTO ANY OF THE DETAILS HERE BECAUSE I'LL BE COVERING THE OPERATING EXPENDITURES IN A LITTLE BIT MORE DETAIL IN A SECOND.

I DID WANT TO SAY THAT WE COMPLETED THE YEAR IN PROCESS AND SUBMITTED OUR REPORTS TO THE STATE CONTROLLER'S OFFICE BOTH ON TIME AND WITHIN BUDGET.

SO NOW REALLY LOOKING AT THE FINAL EXPENDITURES FOR THE '12-'13 FISCAL YEAR AT THE

CATEGORICAL LEVEL. I'M JUST GOING TO HIGHLIGHT SOME VARIANCES BETWEEN BUDGET AND ACTUAL EXPENDITURES OVERALL. WE HAD A BUDGET OF $17.9 MILLION. OUR EXPENDITURES TOTALED $16,304,000, LEAVING A BALANCE OF $1.6 MILLION OR 3 PERCENT. LOOKING AT SOME OF THE BUDGET-TO-EXPENDITURE VARIANCES, OUR EXTERNAL SERVICES, WE HAD SAVINGS THERE OF $732,000, AND THAT REALLY WAS A RESULT OF COSTS THAT EITHER DID NOT MATERIALIZE AT ALL OR DID NOT MATERIALIZE AT THE LEVEL THAT WAS BUDGETED.

COUPLE OF EXAMPLES WOULD BE OUR BOARD COUNSEL. WE HAD SAVINGS OF OVER $200,000 THERE. OUR ANNUAL REPORT WE EXPERIENCED SOME SAVINGS. WE ALSO HAD SOME REDIRECTION OF EXTERNAL SERVICES FUNDS TO EMPLOYEE EXPENSES. SO THAT REALLY WAS A RESULT OF THE MAJORITY OF THE SAVINGS IN EXTERNAL SERVICES.

OUR REVIEW MEETINGS AND WORKSHOPS, WE DID HAVE SAVINGS THERE AS WELL OF $329,000. AND ALTHOUGH WE HELD MOST OF THE MEETINGS, THERE WAS A COUPLE OF WORKSHOPS THAT WE DIDN'T HAVE. WE HAD SAVINGS REALLY IN PART BECAUSE THE STAFF THAT WERE RESPONSIBLE FOR COORDINATING THESE FUNCTIONS WORK CLOSELY WITH THOSE VENDORS THAT WERE WILLING TO GIVE US DISCOUNTS, BETTER RATES. SO THAT RESULTED IN LOWER COST OVERALL.

OUR TRAVEL EXPENSES, WE HAD SAVINGS THERE OF ABOUT $190,000. MOST OF THE COST CENTERS HAD SAVINGS. THE COSTS JUST DID NOT MATERIALIZE AT THE LEVEL THAT WE BUDGETED. BUT IN THAT AREA ALSO WE WORKED WITH OUR TRAVEL AGENCY TO BRING DOWN THE COST. WHAT WE FOUND WAS THAT THERE WAS INTERNET AND NONREFUNDABLE TYPE OF AIRPLANE TICKETS THAT WE COULD PURCHASE THAT WERE ACTUALLY A BETTER COST THAN WHAT WE GET THROUGH THE STATE RATE. SO IN THOSE INSTANCES WHERE IT MAKES SENSE, WE HAVE BEEN TRYING TO SECURE AND USE THOSE RATES SO THAT WE CAN HAVE MORE SAVINGS.

THERE WAS ONE AREA WHERE WE HAD A LITTLE BIT OF OVERAGE, AND THAT WAS IN OUR EQUIPMENT, SUPPLIES, AND SOFTWARE CATEGORY. AND THAT WAS BECAUSE WE WANTED TO MAKE SURE THAT WE HAD UNINTERRUPTED SERVICE FOR OUR COMPUTERS, AND SO WE MADE SOME EQUIPMENT PURCHASES TO STABILIZE OUR SERVERS.

SO NOW LOOKING AT THESE EXPENDITURES BY COST CENTERS, THAT'S THE NEXT CHART, I JUST WANT TO POINT OUT THAT THE MAJORITY OF OUR COST CENTERS, EXPENDITURES WERE BETWEEN 85 TO 90 PERCENT OF WHAT THEY WERE BUDGETED. AS I MENTIONED A SECOND AGO, I.T. WAS SLIGHTLY HIGHER, AND IT WAS DUE TO OUR

SERVER STABILIZATION PURCHASE. AND THEN OUR FINANCE AND OPERATIONS UNIT WAS SLIGHTLY LOWER BECAUSE OF YOU MAY RECALL WE HAD A CHIEF FINANCE OFFICER THAT LEFT IN EARLY AUGUST. SO WE HAD SALARY SAVINGS AND BENEFIT SAVINGS FROM THAT POSITION, AND WE DID EVENTUALLY FILL IT WITH A BUSINESS DEVELOPMENT OFFICER LATER ON IN THE FISCAL YEAR.

SO JUST A QUICK COMPARISON OF THE '12-'13 EXPENDITURES TO THE '11-'12 FISCAL YEAR. OVERALL OUR EXPENDITURES WERE $900,000 MORE IN THE '12-'13 FISCAL YEAR OVER WHAT WE SPENT IN '11-'12 FISCAL YEAR. THE BIGGEST CATEGORY WAS IN EMPLOYEE EXPENSES, AND THAT REALLY WAS DUE TO A VARIETY OF FACTORS. FIRST OF ALL, WE DID HAVE MERIT ADJUSTMENTS OF ABOUT 3 PERCENT. WE ALSO HAD INCREASED BENEFIT COST, WHICH REALLY ARE OUT OF OUR CONTROL. FOR EXAMPLE, THE HEALTH BENEFITS INCREASED IN THE '12-'13 FISCAL YEAR BY ANYWHERE FROM $50 TO $133 A MONTH PER INDIVIDUAL BASED ON THE NUMBER OF DEPENDENTS THAT THEY HAVE. OUR RETIREMENT, THE STATE'S RETIREMENT CONTRIBUTION FOR OUR EMPLOYEES ALSO WENT UP ALMOST 1 PERCENT. AND THEN WE ALSO HAD MORE POSITIONS FILLED DURING THE '12-'13 FISCAL YEAR.

OUR EXTERNAL SERVICES EXPENDITURES ARE

ACTUALLY DOWN FROM THE PRIOR FISCAL YEAR BY ALMOST $1.2 MILLION. THAT'S REALLY BECAUSE WE'VE REDIRECTED SOME SERVICES FROM EXTERNAL SERVICES TO EMPLOYEES. AS YOU MAY RECALL, WE DID THAT LAST YEAR IN OUR BUDGET DEVELOPMENT. WE ALSO HAVE REDUCED SOME OF OUR COST, LIKE I INDICATED, IN OUR LEGAL AREA. ANOTHER AREA WHERE WE'VE HAD SOME DECREASED COST IS IN OUR COMMUNICATION AND OUR OUTREACH EFFORTS BECAUSE WE NOW HAVE STAFF THAT ARE ACTUALLY PERFORMING SOME OF THOSE FUNCTIONS.

AND LET'S SEE. THAT'S PRETTY MUCH ALL THE THINGS THAT I WANTED TO COVER ON THIS COMPARISON.

AND THEN JUST BRINGING US NOW TO THE CURRENT YEAR AND BEYOND. SO FOR THE FIRST MONTH OF THIS FISCAL YEAR WE HAVE DISBURSED $26 MILLION FOR OUR GRANTS. WE CONTINUE TO HAVE A VERY HEALTHY CASH RESERVE, JUST UNDER $63 MILLION. AND THE OFFICE OF THE CHAIR CONTINUES TO WORK WITH THE STATE TREASURER'S OFFICE AND DEPARTMENT OF FINANCE TO ENSURE THAT WE HAVE ADDITIONAL FUNDING EVERY MONTH.

NOW TAKING ALL OF THAT AND PUTTING IT INTO OUR 6-PERCENT CAP, AS YOU KNOW, PROPOSITION 71 SETS ASIDE $180 MILLION FOR OUR GENERAL AND GRANT OPERATIONS. SO AS OF JUNE OF 2013, WE HAVE SPENT 75 OF THAT $180 MILLION. OUR BUDGET IS JUST UNDER $15

MILLION FOR THE CURRENT FISCAL YEAR. SO AT THE END OF THIS FISCAL YEAR, I ANTICIPATE THAT WE'LL BE AT ABOUT HALF OF THAT MONEY SPENT, $90 MILLION, LEAVING US JUST $90 MILLION FOR THE REMAINDER OF OUR OPERATIONS. AND THIS, OF COURSE, ASSUMES THAT WE DON'T GET ANY ADDITIONAL BOND FUNDING OR OTHER FUNDING. AND SO WHAT WE DO THEN IS WE TAKE THAT $90 MILLION AND MAKE SURE THAT WE HAVE SUFFICIENT FUNDINGS TO TAKE US THROUGH THE END OF OUR OPERATIONS.

AND SO BASED ON THAT ASSUMPTION, AS YOU CAN SEE, WE ANTICIPATE THAT WE WILL CONTINUE TO HAVE GRADUAL INCREASES THROUGH THE '16-'17 FISCAL YEAR, WHICH IS THE LAST YEAR THAT WE ANTICIPATE THAT WE WILL BE MAKING GRANT AWARDS. AND THOSE GRADUAL INCREASES INCLUDE AN ASSUMPTION THAT WE WILL HAVE TO START PAYING FOR RENT IN NOVEMBER 2015 AND THEN MINOR COST OF LIVING INCREASES. AND THEN BEGINNING WITH THE '17-'18 FISCAL YEAR AND BEYOND, WE WILL SEE A GRADUAL REDUCTION THROUGH THE '20-'21 FISCAL YEAR.

AND THAT REALLY CONCLUDES MY PRESENTATION. ARE THERE ANY QUESTIONS? NO. OKAY. THANK YOU.

CHAIRMAN THOMAS: THANK YOU, CHILA. I BELIEVE THAT CONCLUDES TODAY'S AGENDA. SO I'D LIKE TO THANK EVERYBODY FOR THEIR PARTICIPATION, AND HAVE

A NICE END OF SUMMER, AND WE WILL SEE EVERYBODY IN BURLINGAME IN OCTOBER. WE STAND ADJOURNED.

(THE MEETING WAS THEN CONCLUDED AT 04:11 P.M.)

160 S. OLD SPRINGS ROAD, SUITE 270, ANAHEIM, CALIFORNIA 92808
1-800-622-6092 1-714-444-4100 EMAIL: DEPO@DEPO1.COM

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 PROCEEDINGS BEFORE THE INDEPENDENT CITIZEN'S OVERSIGHT COMMITTEE OF THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE IN THE MATTER OF ITS REGULAR MEETING HELD AT THE LOCATION INDICATED BELOW

MARRIOTT LA JOLLA
4240 LA JOLLA VILLAGE DRIVE
LA JOLLA, CALIFORNIA
ON
AUGUST 28, 2013

WAS HELD AS HEREIN APPEARS AND THAT THIS IS THE ORIGINAL TRANSCRIPT THEREOF AND THAT THE STATEMENTS THAT APPEAR IN THIS TRANSCRIPT WERE REPORTED STENOGRAPHICALLY BY ME AND TRANSCRIBED BY ME. I ALSO CERTIFY THAT THIS TRANSCRIPT IS A TRUE AND ACCURATE RECORD OF THE PROCEEDING.

BETH C. DRAIN, CSR 7152
BARRISTERS' REPORTING SERVICE
160 S. OLD SPRINGS ROAD
SUITE 270
ANAHEIM, CALIFORNIA
(714) 444-4100