BEFORE THE
SCIENTIFIC AND MEDICAL RESEARCH FUNDING WORKING GROUP
OF THE
CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE
ORGANIZED PURSUANT TO THE
CALIFORNIA STEM CELL RESEARCH AND CURES ACT

REGULAR MEETING

LOCATION: 210 KING STREET
3D FLOOR
SAN FRANCISCO, CALIFORNIA

DATE: SEPTEMBER 19, 2007
10 A.M.

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

BRS FILE NO.: 79292

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1072 S.E. BRISTOL STREET, SUITE 100, SANTA ANA HEIGHTS, CALIFORNIA 92707
PHONE: 714.444.4100 FAX: 714.444.4411 EMAIL: DEPO@DEPO1.COM

SAN FRANCISCO, CALIFORNIA; WEDNESDAY, SEPTEMBER 19, 2007

10 A.M.

DR. SAMBRANO: I THINK WE'RE MISSING JUST A FEW PEOPLE, BUT WE SHOULD PROBABLY GO AHEAD AND GET STARTED. THE FIRST REMINDER TO EVERYBODY IS THAT THIS MEETING IS A PUBLIC MEETING. IT IS BEING TRANSCRIBED, AND SO IT'S

1072 S.E. BRISTOL STREET, SUITE 100, SANTA ANA HEIGHTS, CALIFORNIA 92707
PHONE: 714.444.4100 FAX: 714.444.4411 EMAIL: DEPO@DEPO1.COM

IMPORTANT THAT, BECAUSE WE ARE DOING THIS VIA TELECONFERENCE, THAT EVERYONE STATE THEIR NAME SO THAT THE TRANSCRIBER KNOWS CLEARLY WHO IS SPEAKING AND SO THAT EVERYBODY ELSE HERE ALSO KNOWS WHO IS SPEAKING.

SO, DR. ORKIN, WHENEVER YOU ARE READY.

CHAIRMAN ORKIN: I THINK I'M READY. WE SHOULD CALL THE MEETING TO ORDER. AND ALSO LIKE TO RECOGNIZE THE VICE CHAIR, JOAN SAMUELSON. ARE YOU THERE?

MS. SAMUELSON: YES. HI, STU.

CHAIRMAN ORKIN: HI, JOAN. OKAY. I THINK I'LL HAND IT OVER TO GIL FOR THE ROLL CALL.

DR. SAMBRANO: STARTING OFF WITH THE ROLL CALL. STU ORKIN.

DR. ORKIN: HERE.

DR. SAMBRANO: JOAN SAMUELSON.

MS. SAMUELSON: HERE.

DR. SAMBRANO: ROBERT KLEIN.

MR. KLEIN: HERE.

DR. SAMBRANO: SUSAN BONNER-WEIR.

DR. BONNER-WEIR: HERE.

DR. SAMBRANO: ALI BRIVANLOU.

DR. BRIVANLOU: HERE.

DR. SAMBRANO: JEFF BULTE IS NOT HERE YET. MARIE CSETE.

DR. CSETE: HERE.

DR. SAMBRANO: WHO JUST JOINED, PLEASE?

UNIDENTIFIED SPEAKER: HELLO. I'M CALLING FOR MR. ROBERT KLEIN, WHO IS JOINING.

MR. KLEIN: I'M JUST CHANGING FROM A CELL PHONE TO A HARD LINE.

DR. SAMBRANO: MARCY FEIT. ALEX JOYNER.

DR. JOYNER: HERE.

DR. SAMBRANO: JUDITH KIMBLE.

DR. KIMBLE: HERE.

DR. SAMBRANO: JEFF SHEEHY. JOHN WAGNER, I THINK, WILL JOIN IN ABOUT AN HOUR'S TIME. DAVID WILLIAMS.

DR. WILLIAMS: HERE.

DR. SAMBRANO: JANET WRIGHT. WISE YOUNG.

OKAY. SO THAT'S THE ROLL CALL.

I ALSO WANT TO MAKE SURE EVERYBODY KNOWS WHO IS HERE IN SAN FRANCISCO LIVE. WHO JUST JOINED? DID SOMEBODY JUST JOIN?

DR. WRIGHT: JANET WRIGHT.

DR. SAMBRANO: HI, JANET. THANK YOU.

DR. WRIGHT: GOOD MORNING.

DR. SAMBRANO: SO HERE IN SAN FRANCISCO AT CIRM, WE HAVE SEVERAL CIRM STAFF AND MEMBERS OF THE PUBLIC. WE HAVE DR. RICHARD MURPHY, ARLENE CHIU. WE HAVE KUMAR HARI AND BETTINA STEFFEN, BOTH SCIENTIFIC

OFFICERS. WE HAVE TRICIA CHAVIRA, WHO WILL BE HANDLING THE WEBCAST, AND SO SHE IS HERE. BETH DRAIN, WHO IS OUR TRANSCRIBER, MAYBEL CORTEZ, AND DALE CARLSON, LYNN HARWELL, AND WE HAVE RICK KELLER AND TWO MEMBERS OF THE PUBLIC, ONE WHO IS AN ICOC BOARD MEMBER, DR. OS STEWARD. AND IF YOU COULD IDENTIFY YOURSELF.

MS. HOFSTEADER: I'M MARA HOFSTEADER. I'M THE DIRECTOR OF EDUCATION AND SCIENTIFIC LIAISON FOR THE IRVINE RESEARCH CENTER AND FOR THE STEM CELL CENTER AT UNIVERSITY OF CALIFORNIA IRVINE.

DR. SAMBRANO: TERRIFIC. AND THEN JUST ONE MORE REMINDER BEFORE WE MOVE FORWARD. AGAIN, BECAUSE WE ARE IN A TELECONFERENCE PHONE CALL, IT'S IMPORTANT THAT YOU MUTE WHEN YOU ARE NOT SPEAKING WHENEVER POSSIBLE. AND ALSO PLEASE DO NOT PUT US ON HOLD. OFTENTIMES GREAT, WONDERFUL MUSIC COMES TO OUR EARS WHEN THAT HAPPENS, BUT IT CAUSES US TO HAVE TO ALL HANG UP AND DIAL IN AGAIN. SO NO HOLD AND MUTE WHEN POSSIBLE.

MS. SAMUELSON: ONE MORE THING IF YOU COULD. WHEN PEOPLE IN THE ROOM THERE SPEAK, IF YOU COULD TRY TO HAVE THEM REMEMBER TO GET CLOSE TO A MIC.

DR. SAMBRANO: THANK YOU, JOAN. VERY GOOD POINT.

OKAY. STU, I THINK WE'RE READY TO GO TO THE NEXT ITEM.

CHAIRMAN ORKIN: OKAY. I GUESS I DON'T HAVE MUCH TO SAY IN WELCOME EXCEPT I WANT TO THANK EVERYBODY FOR PARTICIPATING IN THIS MEETING BECAUSE I THINK IT'S IMPORTANT TO CIRM. AND I GUESS, RICH MURPHY, IF YOU'RE THERE, YOU HAVE A FEW MINUTES.

DR. MURPHY: I DO, STU. I'M THERE AND SCHEDULED FOR 15 MINUTES, AND I WILL MAKE IT FIVE.

I AM CURRENTLY SERVING IN A SIX-MONTH TERM AS THE INTERIM PRESIDENT OF CIRM. I WAS ON THE ICOC FROM ITS BEGINNING WHILE I WAS THE PRESIDENT OF THE SALK INSTITUTE, AND I RETIRED FROM THAT POSITION AS OF JULY 1 WITH THE INTENTION OF MOVING BACK TO BOSTON. AND AS I WAS CLEANING OUT THE GARAGE, THE PHONE RANG AND IT WAS BOB KLEIN SAYING, "WOULD YOU SERVE AS INTERIM PRESIDENT UNTIL WE FIND A NEW PRESIDENT FOR THE INSTITUTE?" AND I THOUGHT THAT WOULD BE A GREAT IDEA BECAUSE IT'S AN INTERESTING TIME IN CIRM'S DEVELOPMENT.

AS YOU ALL KNOW, THE LEGAL HURDLES HAVE BEEN OVERCOME. WE ARE NOW CAPABLE OF GOING OUT AND SEEKING BOND FINANCING, WHICH BOB IS DOING, TO THE TUNE OF $250 MILLION, WHICH HOPEFULLY WILL BE AVAILABLE VERY SOON TO FUND OUR PROGRAMS. SO CIRM IS REALLY READY TO SPROUT ITS WINGS AND TO DO ALL THE THINGS THAT WE HOPE IT CAN DO.

MY GOALS IN BEING HERE FOR THIS SHORT TIME WERE REALLY TO ENSURE THAT THE PROGRAMS CONTINUE TO MOVE

FORWARD; AND, OF COURSE, IT MAKES IT MUCH EASIER WITH THE MONEY BEING AVAILABLE TO US. TO LOOK AT KIND OF THE ORGANIZATION OF CIRM TO BE SURE IT'S AS EFFICIENT AND SMOOTH RUNNING AS IT COULD BE, AND REALLY TO TEE UP THE INSTITUTE FOR THE NEXT PERMANENT PRESIDENT, WHO WE THOUGHT WE WOULD BE ABLE TO IDENTIFY OVER THE NEXT SIX MONTHS.

I BEGAN ON SEPTEMBER 6TH AND WORKED SEPTEMBER 6TH AND 7TH, AND THEN LAST WEEK I WAS IN CANADA. BY THE TIME I CAME BACK, A NEW PRESIDENT HAD BEEN SELECTED. I WILL TAKE CREDIT FOR ALL OF THAT. AND, OF COURSE, THE NEW PRESIDENT IS ALAN TROUNSON FROM MONASH UNIVERSITY IN MELBOURNE. AND ALAN WAS WITH US THESE PAST MONDAY AND TUESDAY, AND I THINK EVERYONE IS VERY EXCITED. HE'S AN IDEAL CANDIDATE TO BE RUNNING THE INSTITUTE.

HE'S A STEM CELL SCIENTIST. HE WAS IN THE FIELD AT THE VERY BEGINNING. HE IS A PERSON WHO HAS A GOOD SENSE OF GETTING THINGS FROM THE BENCH TO THE BEDSIDE. HE'S BEEN INVOLVED IN BUSINESS OPPORTUNITIES, STARTED SEVERAL COMPANIES. HE'S WORKED VERY CLOSELY WITH GOVERNMENT, WHICH HAS NOT ALWAYS BEEN EASY IN AUSTRALIA, AND ALSO IN SINGAPORE, AND HE'S A REALLY GOOD GUY, FOR THOSE OF YOU WHO HAVE NOT MET HIM. I THINK HE'S GOING TO BE AN IDEAL PRESIDENT FOR THE INSTITUTE.

SO MY INTENTION IS TO BE HERE AT LEAST UNTIL

ALAN COMES; AND THEN IF ALAN NEEDS ME TO HELP IN THE TRANSITION, I'M MORE THAN HAPPY TO STAY UP TO THE SIX-MONTH PERIOD. SO IT'S AN EXCITING TIME.

I ALSO WANT TO THANK THE COMMITTEE, STU, AND ALL OF THE PEOPLE THAT WORK FOR CIRM. WE REALIZE THAT THE INSTITUTE HAS PUT YOU THROUGH SOME MAJOR HOOPS IN THE SEED COMPETITION AND THE COMPREHENSIVES. AND I THINK CERTAINLY IN CALIFORNIA THE FEELING IS THAT THE PEER REVIEW HAS BEEN NOTHING SHORT OF MAGNIFICENT. AND THAT ONLY HAPPENED BECAUSE OF ALL THE EFFORT THAT ALL OF YOU HAVE PUT IN TOGETHER WITH THE EFFORTS OF THE CIRM STAFF, WHICH HAVE BEEN REMARKABLE.

SO I THANK YOU ON BEHALF OF CERTAINLY THE ICOC, ON BEHALF OF THE CIRM STAFF FOR ALL THAT YOU'VE DONE, WHICH IS ALSO MY WAY OF ASKING YOU TO CONTINUE. AT THIS TIME, ESPECIALLY AS WE HAVE SOME NEW PROGRAMS THAT ARE READY TO GO, WE WILL NEED YOUR CONTINUED GUIDANCE AND ENERGY AND COMMITMENT. AND ON BEHALF OF EVERYONE, I WANT TO THANK YOU FOR DOING IT AND THANK YOU FOR CONTINUING WITH US. SO, STU, THAT'S WHAT I HAVE TO SAY.

CHAIRMAN ORKIN: THANK YOU VERY MUCH. I THANK YOU FOR THE KIND WORDS ABOUT THE COMMITTEE. ALSO I'D LIKE TO CONGRATULATE YOU AND BOB KLEIN ON A GREAT SELECTION AS A PRESIDENT.

DR. MURPHY: THANK YOU.

CHAIRMAN ORKIN: I GUESS, GIL, WE HAVE SOME NEW MEMBERS, DISCUSSION OF THAT.

DR. SAMBRANO: YES. I WILL GET TO THAT. I THINK WE HAD A COUPLE OF PEOPLE WHO MAY HAVE JOINED THE CALL. IF YOU COULD PLEASE IDENTIFY YOURSELVES.

MR. SHEEHY: JEFF SHEEHY AND I'M ON MY WAY DOWN THERE, BUT I WAS HELD UP WITH THE TAXI. I'M ON.

DR. SAMBRANO: THANK YOU, JEFF. WAS THERE SOMEBODY ELSE?

MS. BECKER: PAT BECKER.

DR. SAMBRANO: OKAY. THANK YOU. SO, YES, WE HAVE HAD SOME RECENT ACTIVITY, AND SO I WANT TO JUST REPORT ON THE SCIENTIFIC MEMBERSHIP OF OUR GRANTS WORKING GROUP. AND, AS YOU KNOW, OUR GRANTS WORKING GROUP, IN ADDITION TO THE PATIENT ADVOCATE MEMBERS, CONSISTS OF 15 SCIENTISTS FROM OUTSIDE OF CALIFORNIA THAT ARE APPOINTED BY THE ICOC. IN ADDITION TO THESE REGULAR MEMBERS, THE GRANTS WORKING GROUP ALSO HAS SEVERAL ALTERNATE SCIENTIFIC MEMBERS, ALSO APPOINTED BY THE ICOC, WHO ADD TO THE BREADTH OF EXPERTISE OF OUR GROUP.

WHEN SERVING IN A REVIEW MEETING OR A POLICY MEETING, REGULAR AND ALTERNATE MEMBERS HAVE THE SAME ROLES AND RESPONSIBILITIES AND ARE SUBJECT TO THE SAME POLICIES AND DISCLOSURES. IF A REGULAR MEMBER RESIGNS HIS OR HER POST, THE ICOC MAY APPOINT AN ALTERNATE MEMBER

TO THAT POSITION.

SO I WANT TO REPORT TO YOU THAT UNFORTUNATELY DR. ANDREW FEINBERG RECENTLY RESIGNED HIS POST. AND SO ON AUGUST 8TH, THE ICOC APPOINTED DR. MARIE CSETE FROM EMORY UNIVERSITY AS A REGULAR MEMBER. SO WE WANT TO CONGRATULATE HER ON THAT APPOINTMENT. AND SHE IS JOINING US HERE BY TELECONFERENCE.

DR. CSETE: THANK YOU.

DR. SAMBRANO: IN ADDITION, THE ICOC APPOINTED DR. IAN DUNCAN FROM THE UNIVERSITY OF WISCONSIN AS A FIRST ALTERNATE, WHICH MEANS IF ANOTHER REGULAR MEMBER HAPPENS TO RESIGN THEIR POST, DR. DUNCAN WILL BE FIRST IN LINE TO TAKE THAT POSITION.

I ALSO WANT TO REPORT ON FIVE NEW ALTERNATE MEMBERS THAT THE ICOC APPOINTED. THIS, AGAIN, IN AUGUST. THE FIRST IS DR. RICHARD GOODMAN, AND I WILL JUST GIVE YOU A VERY BRIEF, TWO-SENTENCE DESCRIPTION TO GIVE YOU AN IDEA OF WHO THOSE FOLKS ARE. DR. GOODMAN IS A MEMBER OF THE NATIONAL ACADEMY OF SCIENCES AND THE INSTITUTE OF MEDICINE, A PROFESSOR OF CELL AND DEVELOPMENTAL BIOLOGY AT OREGON HEALTH AND SCIENCE UNIVERSITY. HE SERVES AS THE DIRECTOR OF THE BALDWIN INSTITUTE, WHICH IS A PRIVATELY OWNED RESEARCH UNIT OF THE OREGON HEALTH AND SCIENCE UNIVERSITY THAT SUPPORTS BASIC RESEARCH AND TRAINING PROGRAMS IN AREAS SUCH AS NEUROBIOLOGY, HEARING,

AND GENE EXPRESSION.

WE ALSO HAVE DR. SHELLY HEIMFELD, WHO IS AN ASSOCIATE FACULTY MEMBER AT THE FRED HUTCHINSON CANCER CENTER IN SEATTLE, WASHINGTON, AND IS DIRECTOR OF THE CELLULAR THERAPY LABORATORY AND GMP CELL PROCESSING FACILITY. HE CURRENTLY SERVES AS PRESIDENT ELECT FOR THE INTERNATIONAL SOCIETY OF CELLULAR THERAPY AND IS ON THE BOARD OF DIRECTORS FOR THE FOUNDATION FOR THE ACCREDITATION OF CELLULAR THERAPY.

THE THIRD MEMBER IS DR. MICHAEL D. SCHNEIDER, WHO JUST HAS RECENTLY ASSUMED THE ROLE OF HEAD OF CARDIOVASCULAR SCIENCE AND PROFESSOR OF CARDIOLOGY AT THE NATIONAL HEART AND LUNG INSTITUTE OF THE IMPERIAL COLLEGE IN LONDON. PRIOR TO THIS TRANSITION, HE WAS AT THE M. D. ANDERSON FOUNDATION CHAIR AT BAYLOR COLLEGE OF MEDICINE AND SERVED AS DIRECTOR OF THE CENTER FOR CARDIOVASCULAR DEVELOPMENT THERE.

THE FOURTH IS DR. JOHN R. SLADEK, WHO HAS RECENTLY RETURNED TO THE UNIVERSITY OF COLORADO AS A PROFESSOR OF PEDIATRICS AND NEUROSCIENCE. HE PREVIOUSLY SERVED AS PRESIDENT AND CEO FOR THE CALIFORNIA LUTHERAN UNIVERSITY AND ALSO PREVIOUSLY SERVED AS VICE CHANCELLOR OF RESEARCH AND PROFESSOR OF PSYCHIATRY IN NEUROSCIENCE AT THE UNIVERSITY OF COLORADO AT DENVER HEALTH SCIENCE CENTER.

AND THE LAST MEMBER IS DR. THOMAS P. ZWAKA, WHO IS AN ASSISTANT PROFESSOR IN THE DEPARTMENT OF MOLECULAR AND CELLULAR BIOLOGY AND THE CENTER FOR CELL AND GENE THERAPY AT BAYLOR COLLEGE OF MEDICINE. ALSO SERVES AS DIRECTOR OF THE BAYLOR EMBRYONIC STEM CELL CORE AND WAS ONE OF THE FOUNDERS OF THE STEM CELL AND REGENERATIVE MEDICINE CENTER THERE.

SO I WANT TO CONGRATULATE THOSE NEW MEMBERS. DR. ORKIN, I TURN IT BACK TO YOU.

CHAIRMAN ORKIN: ONE QUESTION THAT MAY RAISE, GIL, IS WITH THE INCREASED -- NOW WITH THE MONEY AVAILABLE AND INCREASED ACTIVITY IN TERMS OF PROGRAMS, ARE THERE ENOUGH MEMBERS OF THE GRANTS WORKING GROUP EVEN WITH THE ALTERNATES? HOW DO YOU PLAN ON ADDRESSING THIS GOING FORWARD?

DR. SAMBRANO: I THINK WE NEED CERTAINLY YOUR CONTINUED HELP IN RECRUITING ADDITIONAL MEMBERS. I THINK YOU'RE RIGHT. IT'S VERY IMPORTANT TO HAVE A BROAD EXPERTISE AS WE MOVE FORWARD WITH RFA'S THAT WILL ENCOMPASS VERY DIFFICULT AREAS OF SCIENCE. SO ANY CONTRIBUTIONS THAT OUR WORKING GROUP MEMBERS CAN GIVE TO US IN TERMS OF POTENTIAL ALTERNATE MEMBERS AND REVIEWERS IS GREATLY APPRECIATED. SO I THINK THIS WILL BE A CONTINUING AND EVOLVING PROCESS.

CHAIRMAN ORKIN: THANK YOU. ARLENE, WILL YOU

DISCUSS THE RFA SCHEDULE?

DR. CHIU: THE NEXT ITEM, I BELIEVE WE'D LIKE TO PRESENT TO EVERYONE AN UPDATE ON PAST CIRM INITIATIVES. I'M SORRY. I'M MISSING THE ELEMENT. THE RFA SCHEDULE. THANK YOU SO MUCH.

I JUST WANTED TO MENTION THAT BECAUSE WE NOW HAVE SOLID -- LOOK FORWARD TO SOLID FUNDING, WE CAN PLAN A LITTLE BIT BETTER UPCOMING INITIATIVES AND A BETTER TIMETABLE, AND WE'RE WORKING ON THAT. WITHOUT GOING INTO VERY MUCH MORE DETAIL, BECAUSE ALL THIS HAS TO BE APPROVED BY THE ICOC, I JUST WANT TO LET THE GRANTS WORKING GROUP KNOW THAT WE HAVE A PRELIMINARY PLAN THAT STAGGERS THE NUMBER OF MEETINGS AND SETS MUCH FIRMER DATES ON WHEN REVIEW MEETINGS WILL TAKE PLACE SO THAT WE CAN PRESENT TO ALL OF YOU THE INITIATIVE IDEAS, THE CONCEPTS, AND DATES, AND WE CAN GET YOUR AVAILABILITY, AND WE CAN BETTER PREPARE FOR UPCOMING REVIEWS FOR 2008 AND HOPEFULLY INTO 2009.

I KNOW THAT YOU'VE BEEN VERY GENEROUS WITH US. EVERY TIME WE COME UP WITH A NEW RFA, WE START ASKING FOR YOUR AVAILABILITY WITH RATHER SHORT NOTICE, AND WE PLAN TO CHANGE THAT APPROACH SOON. AND THAT BASICALLY WAS THE UPDATE. IF THERE ARE ANY QUESTIONS, I'D BE HAPPY TO ENTERTAIN THEM.

MS. SAMUELSON: ARLENE, THIS IS JOAN. TO ADD

TO THAT, ON BEHALF OF THE ICOC, AS A MEMBER OF IT, IT WOULD BE GREAT FOR ANY WORKING GROUP MEMBER TO PASS ALONG ANY FEEDBACK YOU MIGHT HAVE AS TO WHAT HAS WORKED WITH THE SYSTEM AND WHAT HAS BEEN BURDENSOME. IN MOST CASES, PROBABLY MAYBE ALL, I'D SAY WE'RE ACUTELY AWARE OF THOSE THINGS NEEDED TO PUSH THE BALL AHEAD WITH LIMITED MONEY. BUT GIVEN THOSE OBSTACLES ARE OUT OF THE WAY, WE WANT TO DO IT RIGHT. SO IF THERE'S ANYTHING WE'RE NOT THINKING OF, WE'D LOVE YOUR INPUT.

DR. CHIU: THAT'S EXACTLY RIGHT, JOAN. WE WOULD LIKE TO HEAR FROM ALL WORKING GROUP MEMBERS OF IDEAS OF HOW TO MAKE THIS PROCESS MOVE BETTER. WE ALSO, AS IN, I THINK, ONE PAST MEETING, WE WOULD LIKE TO, IF POSSIBLE, HAVE SORT OF INFORMAL LUNCHEON DISCUSSIONS WITH WORKING GROUP MEMBERS WHO ARE PRESENT AT A REVIEW TO BRAINSTORM AND CRITIQUE THE REVIEW PROCESS CERTAINLY FROM YOUR POINT OF VIEW OF HOW WE CAN MAKE THE WORKINGS OF IT LESS ONEROUS AND SMOOTHER AND TO ACCOMMODATE YOU ALL BETTER.

SO PLEASE THINK ABOUT IT AND SEND US, GIL OR MYSELF, E-MAILS IF YOU HAVE ANY THOUGHTS ABOUT HOW TO MAKE THIS WORK BETTER FOR ALL OF YOU.

MR. KLEIN: ARLENE, THIS IS BOB KLEIN. I THINK THAT IF DR. MURPHY IS STILL THERE, THERE WERE -- IN THE PAST THERE WAS SOME QUESTION WHERE OUR TRAVEL

ARRANGEMENTS WERE BEING MADE, AND THERE WAS TOO MUCH OF A FOCUS ON WHETHER THERE COULD BE MONEY SAVED BY INDIRECT FLIGHTS BECAUSE OF STATE TRAVEL PREFERENCES AND STATE REGULATIONS. AND WE HAVE THE ABILITY AND HAVE TO RECOGNIZE THE ABILITY TO MAKE SURE WE HAVE DIRECT FLIGHT BOOKINGS FOR EVERYONE SO THAT TIME, WHICH IS CRITICAL TO EVERY ONE OF THE REVIEWERS IN THE LOGISTICS PROCESS OF COMING TO MEETINGS, IS MINIMIZED AND DIRECT FLIGHTS ARE ROUTINELY BOOKED WITHOUT UNNECESSARY TRANSFERS.

DR. MURPHY: I MEAN THAT ONLY MAKES SENSE, BOB. FOR THE TALENT WE HAVE, TO BE SHUTTLING THEM OFF TO SMALL AIRPORTS DOESN'T MAKE SENSE. SO WE WILL LOOK INTO WHAT WE CAN DO IN THAT, BUT OBVIOUSLY WE ALL AGREE WITH WHAT YOU'VE JUST SAID.

CHAIRMAN ORKIN: THANK YOU, EVERYONE AND ARLENE. I THINK HAVING BETTER LEAD-TIME FOR THE MEETINGS WILL BE OF GREAT ASSISTANCE, I THINK, IN PLANNING AHEAD.

NEXT WE HAVE GIL WILL DISCUSS, FIRST, ONE OF THE PAST INITIATIVES AND THE SCHOLARS MEETING.

DR. SAMBRANO: YES. I THINK ALSO SOMEBODY MAY HAVE JOINED THE CALL. IF THEY COULD IDENTIFY THEMSELVES, PLEASE. DID SOMEBODY JUST JOIN? OKAY. DID SOMEBODY JUST JOIN NOW?

DR. BULTE: I'M SORRY. IT'S JEFF BULTE, JOHN HOPKINS UNIVERSITY.

DR. SAMBRANO: THANK YOU FOR CALLING IN.

SO I'M GOING TO GIVE A BRIEF REPORT ON THE TRAINING PROGRAM AND THE SCHOLARS MEETING WHICH WE JUST HAD. AND THIS IS THE FIRST OF THE PASSED CIRM INITIATIVES THAT BOTH ARLENE AND I WILL DISCUSS.

SO WITH THE TRAINING PROGRAM, IN APRIL OF 2006, THE CIRM AWARDED TRAINING GRANTS TO 16 CALIFORNIA NONPROFIT AND ACADEMIC INSTITUTIONS WITH THE INTENT TO FOSTER TRAINING OF PREDOCTORAL, POSTDOCTORAL, AND CLINICAL FELLOWS IN THE AREA OF STEM CELL RESEARCH. THIS WAS THE FIRST INITIATIVE OF THE CIRM. IT WAS THE FIRST REVIEW FOR THIS GRANTS WORKING GROUP. IT WAS ALSO THE FIRST APPROVAL AND AWARD BY THE ICOC. SO IT WAS A NUMBER OF FIRSTS FOR US.

EACH INSTITUTION THAT WAS SELECTED BRINGS UNIQUE STRENGTHS IN AREAS OF SPECIALIZATION IN STEM CELL RESEARCH AND TO THE TRAINING. AND OVERALL THE PROGRAM SUPPORTS UP TO 170 TRAINEES. AND THIS MONTH WE ARE HOLDING THE FIRST ANNUAL CIRM SCHOLARS MEETING TO SEE THESE TRAINEES. THE PURPOSE IS TO OFFER THE CIRM SCHOLARS AND THE TRAINEES THE OPPORTUNITY TO PRESENT THEIR PRELIMINARY DATA, TO ENGAGE IN SCIENTIFIC DISCUSSION, AND EXCHANGE IDEAS. IT'S AN OCCASION TO MEET WITH THEIR PEERS AND MENTORS ACROSS DIFFERENT INSTITUTIONS, TO SHARE AND DISCOVER WHAT OTHERS ARE

WORKING ON AS WELL.

AND, OF COURSE, FOR THE CIRM, IT'S AN OPPORTUNITY TO SEE FIRSTHAND THE PRODUCT OF OUR TRAINING PROGRAM AND OUR FIRST RFA. AND BECAUSE WE HAVE SUCH A LARGE NUMBER OF TRAINEES AND ASSOCIATED MENTORS AND PROGRAM DIRECTORS, WE HAVE SPLIT THE MEETING INTO TWO. WE HAVE A NORTHERN CALIFORNIA MEETING AND A SOUTHERN CALIFORNIA MEETING. THE NORTHERN COHORT ALREADY MET ON SEPTEMBER 11TH, AND THE SOUTHERN COHORT WILL MEET NEXT WEEK ON SEPTEMBER 28TH.

BOTH MEETINGS ARE ESSENTIALLY FOLLOWING A SIMILAR AGENDA. THEY FEATURE SHORT TALKS WHICH ARE GIVEN BY ONE SELECTED TRAINEE FROM EACH INSTITUTION THAT'S REPRESENTED AT THE MEETING. THERE IS A POSTER SESSION TO DISPLAY AND DISCUSS RESEARCH ON A ONE-TO-ONE BASIS. AND OVERALL WE GOT 115 ABSTRACTS OVER BOTH MEETINGS, SO WE GOT A VERY GOOD RESPONSE AND INTEREST.

AND, IN ADDITION, WE HAVE ONE FINAL ELEMENT, SCHOLARS LEADING AND PARTICIPATING IN DISCUSSION GROUPS ON SPECIFIC TOPICS, SUCH AS TRANSLATIONAL CHALLENGES, DIRECTED DIFFERENTIATION, AND CAREER TRANSITIONS. AND THESE ARE RATHER INFORMAL. IT ALLOWS THEM TO LEAD A SMALL GROUP. AND BASED ON WHAT WE HAVE OBSERVED DURING THE FIRST MEETING ON SEPTEMBER 11TH, IT WAS ACTUALLY QUITE IMPRESSIVE. THEY WERE VERY ENTHUSIASTIC IN THEIR

PARTICIPATION, AND I THINK THE QUALITY OF THE TALKS AND POSTERS WERE CERTAINLY FIRST-RATE.

SO OUR GOAL OVERALL WITH THESE MEETINGS AND THE TRAINING PROGRAM IS TO DEVELOP A NETWORK AND A COMMUNITY OF SCIENTISTS IN STEM CELL RESEARCH IN CALIFORNIA. AND I THINK THIS WAS A VERY GOOD FIRST STEP.

SO I GUESS -- AND WE ALSO, AS ARLENE IS REMINDING ME, WE PUBLISH AN ANNUAL CIRM SCHOLARS ABSTRACT BOOK WHICH HAS ALL 115 ABSTRACTS AND GIVES A LITTLE BIT OF BACKGROUND ON THE MEETING AND PROGRAMS. SO IF ANY OF YOU ARE INTERESTED IN SEEING IT, PLEASE LET ME KNOW AND I'D BE HAPPY TO SEND THAT TO YOU.

MR. KLEIN: GIL, THIS IS BOB KLEIN. YOU MIGHT WANT TO COMMENT THAT AS THESE SCHOLARS, A FEW OF WHOM HAVE ACTUALLY NOW BEEN ON THIS FOR MORE THAN A YEAR OR A FULL YEAR, BEGIN TO PUBLISH THEIR PAPERS, I THINK IT'S ABOUT THREE WEEKS AGO NOW ONE OF THESE SCHOLARS, WHO IS A DOCTORAL STUDENT AT UC SAN FRANCISCO, PUBLISHED A PAPER IN *NATURE* AND WAS ON THE COVER OF *NATURE*; IS THAT CORRECT?

DR. SAMBRANO: YES, THAT'S CORRECT. SO WE ARE FORTUNATE TO HEAR EVERY ONCE IN A WHILE FEATURED ARTICLES FROM OUR CIRM SCHOLARS. SO IT'S GREAT TO SEE THAT THEY ARE ACTIVE AND PARTICIPATING, AND WE CERTAINLY ENCOURAGE THEM TO LET US KNOW HOW THEY ARE DOING. CERTAINLY AFTER

A YEAR'S TIME, WE'RE CLEARLY SEEING A LOT OF PARTICIPATION AND A LOT OF RESEARCH IN STEM CELL BIOLOGY THAT IS HAPPENING NOW.

CHAIRMAN ORKIN: THANK YOU, GIL. ANY OTHER COMMENTS? IF NOT, ARLENE, I THINK YOU'RE UP FOR TELLING US ABOUT THE HUMAN ES CELL RESEARCH INITIATIVE.

DR. CHIU: RIGHT. JUST AS A REMINDER, CIRM CAME INTO BEING IN LARGE PART BECAUSE CALIFORNIANS UNDERSTOOD, FIRST, THAT HUMAN EMBRYONIC STEM CELL RESEARCH IS UNDERFUNDED IN THE UNITED STATES, AND NEW IDEAS CANNOT BEAR FRUIT WITHOUT FUNDING THE RESEARCH.

AND, ALSO, THAT NEW INVESTIGATORS WERE KIND OF AFRAID, YOU MIGHT SAY, TO COME INTO SUCH A CONTROVERSIAL FIELD WITHOUT MUCH SUPPORT BEHIND IT. THE RESTRICTIONS IN THE FEDERAL POLICY CAUSED LABS AND EQUIPMENT FUNDED BY THE FEDERAL GOVERNMENT TO NOT BE AVAILABLE TO WORK ON LINES THAT WERE NOT APPROVED BY THE PRESIDENT. AND THE NUMBERS OF THESE UNAPPROVED, BRAND NEW, NOVEL LINES DERIVED WITH NEW TECHNOLOGY ARE GOING UP DAILY.

SO TO ADDRESS THESE NEEDS, LAST YEAR THE CIRM EMBARKED ON AN INITIATIVE TO MEET ALL THESE NEEDS OF THE EMBRYONIC STEM CELL FIELD BY JUMP-STARTING HUMAN EMBRYONIC STEM CELL RESEARCH IN CALIFORNIA. AND THE INITIATIVE THAT WAS LAUNCHED CONSISTED OF THREE NEW RFA'S THAT MANY OF YOU ARE FAMILIAR WITH BECAUSE YOU MIGHT HAVE

REVIEWED ONE OR MORE OF THE APPLICATIONS COMING FROM THESE RFA'S IN THE PAST FEW MONTHS. BUT I'LL PROVIDE A VERY BRIEF UPDATE OF WHAT HAPPENED AFTER THE WORK OF THE GRANTS WORKING GROUP.

SO THE THREE RFA'S CONSISTED OF THE FIRST ONE BEING ONE FOR A SEED GRANT PROGRAM, THE LEON THAL SEED GRANT PROGRAM, NAMED AFTER ONE OF OUR BOARD MEMBERS WHO DIED UNTIMELY DUE TO AN AIRPLANE ACCIDENT THIS YEAR.

THE IDEA OF THE SEED GRANT PROGRAM WAS TO INVITE NEW IDEAS AND APPROACHES SUCH AS PILOT STUDIES TO INVITE INVESTIGATORS NEW TO THE STEM CELL FIELD INTO THIS AREA OF RESEARCH AND TO BRING IN NEWLY INDEPENDENT YOUNG INVESTIGATORS. WE RECEIVED OVER 300 LETTERS OF INTENT TO THIS INITIATIVE, AS SOME OF YOU MAY GROANINGLY REMEMBER, AND 231 COMPLETE APPLICATIONS. AND THE GRANTS WORKING GROUP WENT THROUGH A MARATHON SESSION IN NOVEMBER OF LAST YEAR AND RECOMMENDED A NUMBER OF THESE TO THE ICOC.

THE ICOC WAS VERY, VERY EXCITED ABOUT THESE APPLICATIONS AND APPROVED 74 OF THEM FOR FUNDING IN EARLY 2007 TO A TOTAL COST OF ABOUT 45 AND A HALF, $46 MILLION TO SUPPORT THIS PROGRAM. SO THAT IS THE SEED GRANT PROGRAM THAT MANY OF YOU REVIEWED FOR CIRM.

THE NEXT RFA IS A COMPREHENSIVE RESEARCH PROGRAM. THAT WAS ALSO POSTED IN AUGUST OF LAST YEAR. THE IDEA WAS TO SUPPORT ESTABLISHED INVESTIGATORS IN THE

STEM CELL FIELD TO PROVIDE SOMEWHAT LONGER TERM SUPPORT, UP TO FOUR YEARS, FOR SIGNIFICANT PROJECTS. AND YOU REVIEWED -- THE GRANTS WORKING GROUP REVIEWED THESE APPLICATIONS IN JANUARY.

SO 74 LETTERS OF INTENT WERE RECEIVED FOLLOWED BY 70 COMPLETE APPLICATIONS. AFTER THE JANUARY REVIEW, 29 OF THE 70 APPLICATIONS WERE APPROVED BY THE ICOC IN MARCH, I BELIEVE, OF THIS YEAR TO A TUNE OF $74 AND A HALF MILLION.

THE LAST LEG OF THIS THREE-PRONG ATTACK IS TO PROVIDE NIH-FREE SPACE AND EQUIPMENT. AND THE RFA IS ENTITLED "SHARED RESEARCH LABS AND STEM CELL TECHNIQUES COURSE PROGRAM FOR HUMAN EMBRYONIC STEM CELLS." THIS WAS POSTED, I BELIEVE, IN JANUARY OF THIS YEAR. WE MOVED RATHER QUICKLY FORWARD. THIS IS AN INITIATIVE THAT WAS REVIEWED BOTH FOR SCIENTIFIC MERIT AND BY THE FACILITIES WORKING GROUP FOR TECHNICAL EXCELLENCE OF THEIR PROPOSED PLAN BECAUSE WE WERE FUNDING RENOVATION OF SPACE AND EQUIPMENT TO CONDUCT RESEARCH, PROVIDE THREE YEARS OF SUPPORT FOR OPERATIONS AND MAINTENANCE. AND FOR THE COURSE PART OF IT, IF THEY SHOULD CHOOSE TO APPLY, SOME MORE EQUIPMENT AND PERHAPS MINOR RENOVATION AND ALSO FUNDING FOR TEACHING TECHNIQUES FOR THE DERIVATION AND CULTURING OF HUMAN EMBRYONIC STEM CELLS.

CIRM RECEIVED 25 LETTERS OF INTENT, 22 COMPLETE

APPLICATIONS. PART 1, THE SCIENTIFIC PART, WAS REVIEWED BY THE GRANTS WORKING GROUP IN FEBRUARY OF THIS YEAR. PART 2, THE TECHNICAL PLANS, WERE REVIEWED BY THE FACILITIES WORKING GROUP IN MARCH. AND IN JUNE OF THIS YEAR, THE ICOC APPROVED 17 OF THESE APPLICATIONS FOR FUNDING; AND OF THE 17, 6 ALSO RECEIVED APPROVAL FOR FUNDING FOR TEACHING TECHNIQUES COURSES. AND THE COMPLETE FUNDING FOR THIS WHOLE PROGRAM, SHARED LABS AND TECHNIQUES COURSES, CAME TO $50 MILLION.

AND THAT IS THE UPDATE ON THE THREE-PRONG INITIATIVE TO JUMP-START HUMAN EMBRYONIC STEM CELLS. ARE THERE ANY QUESTIONS?

CHAIRMAN ORKIN: ANY QUESTIONS? I'D JUST LIKE TO MAKE A COMMENT -- THIS IS STU AGAIN -- THAT THE QUALITY OF THE APPLICATIONS IN THE SEED GRANT PROCESS AS WELL AS THE COMPREHENSIVE, PARTICULARLY THE SEED GRANT, FAR EXCEEDED WHAT WE MIGHT HAVE IMAGINED. THEY'RE REALLY QUITE EXCELLENT.

DR. CHIU: I WILL ALSO MENTION THAT WE'RE WORKING ON A COMPENDIUM OF THE AWARDS PUT OUT BY CIRM, AS WELL AS APPLICATIONS THAT HAVE BEEN APPROVED FOR FUNDING BY THE ICOC. THAT SHOULD BE READY IN OCTOBER, AND WE WILL BE PUBLISHING THAT ON THE WEB. AND WE WILL LET YOU ALL KNOW SO THAT YOU CAN SEE WHICH ONES FINALLY GOT APPROVAL FOR FUNDING.

DR. MURPHY: STU, RICH MURPHY. THAT'S AN INTERESTING COMMENT YOU MADE. WHY DO YOU THINK THE SEED'S WERE MORE COMPELLING THAN THE COMPREHENSIVES?

CHAIRMAN ORKIN: IT'S HARD TO SAY. I THINK IT MAY HAVE BEEN THAT IT WAS THE -- SORT OF THE FIRST PASS, IF YOU WILL, OF APPLICATIONS. IT ALSO WAS THAT THEY WERE FROM YOUNGER INVESTIGATORS WHO MAY HAVE BEEN MORE BOLD IN WHAT THEY'RE PUTTING FORWARD. SOME OF THE COMPREHENSIVE GRANTS WERE FROM MORE ESTABLISHED INVESTIGATORS WHO TRIED TO PLAY IT SAFE PERHAPS.

DR. MURPHY: THANK YOU.

CHAIRMAN ORKIN: EVEN THE COMPREHENSIVES WERE QUITE GOOD ON THE WHOLE.

THE NEXT TOPIC IS CURRENT CIRM INITIATIVES. AND KUMAR HARI WILL TALK ABOUT THE NEW FACULTY AWARD INITIATIVE.

DR. HARI: THANKS, STU. AND GOOD MORNING EVERYONE OR GOOD AFTERNOON, DEPENDING ON YOUR LOCATION. I WANT TO GIVE YOU A BRIEF UPDATE ABOUT OUR CURRENT RFA. IN LATE JUNE WE POSTED THIS RFA FOR NEW FACULTY AWARDS. AND THESE ARE DESIGNED TO SUPPORT M.D. AND PH.D. SCIENTISTS IN THEIR EARLY YEARS AS INDEPENDENT LEAD INVESTIGATORS AND FACULTY MEMBERS. AND THE GOAL HERE IS REALLY TO DEVELOP THE NEXT GENERATION OF SCIENTIFIC AND CLINICAL STEM CELL RESEARCH LEADERS HERE IN THE STATE OF

CALIFORNIA.

SO RELATIVE TO THE EARLIER PROGRAMS THAT ARLENE DESCRIBED FOR YOU, THIS IS A RATHER NEW RFA FOR US IN A COUPLE OF WAYS. FIRST, WHILE PREVIOUS CIRM RESEARCH GRANTS HAVE FOCUSED ON HUMAN EMBRYONIC STEM CELL RESEARCH, THE NEW FACULTY AWARDS ARE GOING TO SUPPORT RESEARCH ACROSS THE FULL RANGE OF STEM CELL TYPES. THAT'S HUMAN AND ANIMAL, ADULT AND EMBRYONIC, MODEL SYSTEMS. ALL TYPES ARE OPEN FOR APPLICATION.

SECOND, THIS PARTICULAR RFA HAS TWO CATEGORIES OF AWARD. ONE THAT IS OPEN GENERALLY TO ANY NEW FACULTY, AND ANOTHER THAT IS DIRECTED TOWARDS PHYSICIAN/SCIENTISTS. SO THOSE ARE INDIVIDUALS WHO HAVE COMPLETED MEDICAL TRAINING IN A RESIDENCY PROGRAM. WE'D REALLY LIKE TO BRING THAT TRANSLATIONAL FOCUS INTO CIRM AND SUPPORT THAT FOCUS WITHIN THE STATE OF CALIFORNIA.

SO ANOTHER ASPECT OF THIS PARTICULAR AWARD IS THAT WE REQUESTED THAT INSTITUTIONS NOMINATE INDIVIDUALS FOR THESE AWARDS AND, IN FACT, LIMITED THE NUMBER OF APPLICATIONS PER INSTITUTION TO GET THE VERY BEST AND BRIGHTEST. AND WE WERE TOLD THAT THE INTERNAL COMPETITIONS WERE ACTUALLY RATHER INTENSE, SO WE CAN BE ASSURED THAT WE ARE GETTING SOME OF THE BEST APPLICANTS OUT THERE.

THESE APPLICATIONS WILL BE EVALUATED IN THREE

AREAS. THOSE ARE, FIRST, THE SIGNIFICANCE, INNOVATION, DESIGN, AND FEASIBILITY OF THE RESEARCH PLAN. SECOND, THE QUALIFICATIONS AND CAREER DEVELOPMENT PLAN OF PRINCIPAL INVESTIGATORS AS THESE ARE RELATIVELY LONG-TERM. THAT'S FIVE-YEAR AWARDS. AND THIRD, THE COMMITMENT AND TRACK RECORD OF THE INSTITUTION ITSELF. SO CIRM REALLY WANTS TO SEE THAT THE INSTITUTION IS SUPPORTING THESE YOUNG INVESTIGATORS AND, IN FACT, PARTNERING WITH CIRM WITH RESPECT TO BRINGING THEM ALONG IN THEIR CAREERS.

SO APPLICATIONS FOR THESE AWARDS WERE DUE ON AUGUST 30TH, AND WE RECEIVED 59 TOTAL APPLICATIONS. OUR BOARD, THE ICOC, HAS APPROVED THE FUNDING OF UP TO 15 OF THE BASIC NEW FACULTY AWARDS AND 10 OF THE PHYSICIAN/SCIENTIST AWARDS. OUR REVIEW IS SET FOR OCTOBER 23D AND 24TH, AND WE HOPE TO BRING THE RECOMMENDATIONS OF THE GRANTS WORKING GROUP TO THE ICOC IN DECEMBER.

SO I'LL TAKE ANY QUESTIONS IF THERE ARE ANY.

DR. BRIVANLOU: KUMAR, THIS IS ALI BRIVANLOU FROM THE ROCKEFELLER. I JUST HAVE A VERY QUICK QUESTION ABOUT THE RATIONALE OF EXTENDING THE YOUNG INVESTIGATOR AWARDS TO INCLUDE OTHER STEM CELLS OUTSIDE OF HUMAN EMBRYONIC STEM CELLS. IF I UNDERSTAND CORRECTLY FROM THE RATIONALE OF CIRM, ALSO THE WAY IT WAS PRESENTED BY

ARLENE A FEW MINUTES AGO, THE RATIONALE WAS THAT IT WAS A FEELING THAT THE FEDERAL FUNDS WERE NOT BEING USED AND WERE UNDERFUNDING STEM CELL RESEARCH. I BELIEVE THAT THIS IS THE CASE IN OTHER AREAS OUTSIDE OF HUMAN EMBRYONIC STEM CELLS. SO I'M WONDERING WHAT THE RATIONALE FOR EXPANDING THIS, KNOWING THAT MANY OTHER FUNDING AGENCIES, NOT ONLY FEDERAL, BUT ALSO PRIVATE, CAN COVER BEAUTIFULLY IN SELECTING THE OTHER FIELDS OF STEM CELLS.

DR. HARI: HERE WITH THESE PARTICULAR AWARDS, IT'S NOT CLEAR OR WE CAN'T PREDICT PRECISELY WHERE THE INNOVATIONS ARE GOING TO COME FROM. SO IN TERMS OF SUPPORTING THE BREADTH OF BUILDING A FOUNDATION OF SCIENTIFIC EXCELLENCE WITHIN THE STATE, WE DECIDED TO OPEN THIS PARTICULAR RFA UP TO ANY TYPE OF STEM CELL SO THAT WE CAN CAPTURE THE REAL BREADTH OF EXPERTISE AND INTELLECT WITHIN THE STATE.

DR. BRIVANLOU: RIGHT. AS YOU SAID, ALSO, I THINK THERE IS SELF-CENSORSHIP GOING ON AMONG SCIENTISTS THAT ARE A LITTLE BIT AFRAID OF ENTERING THE FIELD BECAUSE OF THE RESTRICTIONS THAT WE'VE ALREADY MENTIONED. DO YOU THINK THAT THIS IS GOING TO ENRICH FOR CANDIDATES WHO ARE ACTUALLY NOT GOING TO BE WORKING ON HUMAN EMBRYONIC STEM CELLS? AND HOW WOULD CIRM REACT IF 95 PERCENT OF THE APPLICATIONS YOU GET ARE COMPLETELY NOT ON

HUMANS?

DR. CHIU: MAY I TRY TO ADDRESS THAT, ALI? I THINK YOU BROUGHT UP A VERY IMPORTANT POINT, THAT OUR MAIN FOCUS IS HUMAN EMBRYONIC STEM CELLS. WE ALSO KNOW FROM THE COMMUNITY THAT A LOT OF FINDINGS OR APPROACHES IN HUMAN EMBRYONIC STEM CELLS ARE BASED ON FINDINGS THAT ARE EMERGING FROM OTHER DEVELOPMENTAL BIOLOGY STEM CELL RESEARCH AREAS. AND WE WANTED TO BUILD THIS CADRE OF LEADERS IN CALIFORNIA, AND WE HOPE THAT THEY WILL NETWORK, OF COURSE, SO THAT WE CAN LEVERAGE BOTH THE HUMAN EMBRYONIC STEM CELL RESEARCH FIELD AS WELL AS INFORMATION FROM OTHER REGENERATIVE MEDICINE APPROACHES.

AND THE SECOND REASON IS FOR YOUNG FACULTY MEMBERS, WE'VE BEEN HEARING THAT THE CURRENT FUNDING LEVELS AT THE NIH HAS REALLY DISCOURAGED PEOPLE FROM BEING LEADERS IN THE FIELD OR EVEN ENTERING THE FIELD. AND FOR THOSE THAT INSTITUTIONS HAVE MADE A COMMITMENT TO, WE REALLY WANTED TO SUPPORT THAT.

BY RESTRICTING TO FOUR APPLICATIONS PER INSTITUTION, WE HOPE TO HELP SUPPORT THE BEST AND THE BRIGHTEST. WE ALSO WANTED TO SEND A MESSAGE OUT TO THE INSTITUTIONS THAT WE WANTED THEM TO PARTNER AND THAT THEY NEED TO MENTOR THEIR YOUNG FACULTY AND HELP THEM BECOME SUCCESSFUL. SO IT WAS SORT OF A THREE-PRONG ATTACK ON THIS LEVEL. WE'RE DOING THE TRAINING. WE'VE DONE THE

SEED'S FOR TWO YEARS, THE COMPREHENSIVES. NOW IT'S THE YOUNG INVESTIGATORS WHO HAVE MADE A COMMITMENT TO RESEARCH, WE WANT TO HELP THEM TO BE SUCCESSFUL. SO IT WAS WITH THAT IN MIND.

DR. BRIVANLOU: I APPRECIATE VERY MUCH. YOU KNOW, MOST OF US ARE MODEL SYSTEMS OF DEVELOPMENTAL BIOLOGIES WORKING WITH FROGS. AND CERTAINLY THE ISSUE IS NOT THAT THOSE ARE NOT IMPORTANT OR THEY SHOULD NOT BE FOLLOWED. MY WORRY IS THAT IT CREATES A PATH OF LESS RESISTANCE FOR THE YOUNG INVESTIGATORS TO TARGET MORE MODEL SYSTEMS THAN HUMAN SYSTEMS. ALL I'M SAYING IS PERHAPS AN AMENDMENT CAN BE MADE TO SAY IN ADDITION TO THE HUMAN EMBRYONIC STEM CELL. IF A LABORATORY WANTS TO DO A COMPARATIVE WORK IN MODEL STEM CELLS, THAT THEY WOULD BE FUNDED.

DR. CHIU: YOU WILL BE PART OF THE REVIEW PROCESS, AND YOUR RECOMMENDATIONS WILL BE TAKEN VERY SERIOUSLY DURING PROGRAMMATIC REVIEW, AND YOUR COMMENTS WILL CARRY A LOT OF WEIGHT TO THE ICOC.

DR. BRIVANLOU: THANK YOU.

DR. WILLIAMS: THIS IS DAVE WILLIAMS. I'VE JUST GOT ONE SMALL SORT OF OPERATIONAL QUESTION. ON PAGE 3, UNDER PRINCIPAL INVESTIGATOR ELIGIBILITY, THERE'S A SENTENCE THAT SAYS, "AT ACADEMIC INSTITUTIONS, INDEPENDENT INVESTIGATORS ARE EXPECTED TO BE TENURE-TRACK

FACULTY POSITIONS." HAVING SERVED ON REVIEW COMMITTEES FOR A LOT OF DIFFERENT THINGS, THIS AREA FOR JUNIOR FACULTY AWARDS ALWAYS IS AN AREA THAT CAN CAUSE CONFUSION.

I WONDER WHY THE WORDING WAS THAT WAY RATHER THAN MORE EXPLICITLY, THAT TO BE ELIGIBLE, YOU HAD TO BE A TENURE-TRACK FACULTY MEMBER. OR IS THAT WHAT YOU INTENDED, IN FACT?

DR. HARI: DR. WILLIAMS, THE INTENT HERE IS REALLY TO CAPTURE INVESTIGATORS WHO MAY BE EMPLOYEES OR WORKING AT PRIVATE INSTITUTIONS. AND WE HAD TO GIVE PEOPLE A SENSE AT THOSE INSTITUTIONS OF THE TYPES OF INDIVIDUALS WE WERE LOOKING FOR BECAUSE REALLY THE TITLES OF EMPLOYMENT AT THOSE RESEARCH INSTITUTIONS ARE SO VARIED, THAT WE COULDN'T GIVE STRICT DEFINITIONS FOR WHO MIGHT BE ELIGIBLE. SO BEYOND SAYING THAT AN INDIVIDUAL MUST BE WITHIN THE FIRST SIX YEARS OF THEIR FIRST INDEPENDENT -- START DATE OF THEIR FIRST INDEPENDENT POSITION, WE HAD TO GIVE THEM ALSO A SENSE OF THE TYPE OF COMMITMENT WE EXPECTED TO SEE FROM THE INSTITUTIONS. THAT WAS THE INTENT BEHIND THAT PHRASING.

DR. WILLIAMS: WHAT'S THE ANSWER TO THE QUESTION IF YOUR ACADEMIC INSTITUTION, DOES THAT MEAN IF YOU ARE AN INSTRUCTOR ON A NONTENURE TRACK ACADEMIC TRACK, ARE YOU ELIGIBLE OR NOT ELIGIBLE?

DR. CHIU: NOT ELIGIBLE.

DR. HARI: YOU WOULDN'T BE ELIGIBLE.

DR. WILLIAMS: I DON'T KNOW ABOUT EVERYBODY ELSE, BUT I WONDER IF YOU SHOULD BE MUCH MORE EXPLICIT ABOUT THIS REQUIREMENT THAN WHAT THIS APPEARS TO BE?

CHAIRMAN ORKIN: I THINK IF THE INSTITUTIONS ARE SELECTING -- PRESELECTING THOSE CANDIDATES THEY WANT TO GO FORWARD, THEY'RE PROBABLY NOT GOING TO SELECT THE INSTRUCTORS WHO ARE IN THIS KIND OF NEBULOUS AREA. OR IF THEY DO, THEN THE COMMITTEE CAN DECIDE DISPOSITION AT THAT TIME.

DR. HARI: I CAN TELL YOU THAT AT THE LOI SUBMISSION STAGE FOR THIS RFA, WE DID RECEIVE A COUPLE OF PHONE CALLS FROM APPLICANTS AND INSTITUTIONS REGARDING THE ELIGIBILITY OF PARTICULAR INDIVIDUALS. FOR PEOPLE IN THE GRAY ZONE, WE DID LET THEM KNOW THAT THE COMMITMENT OF THE INSTITUTION, BOTH DEMONSTRATED AS WELL AS FUTURE, IS A KEY COMPONENT OF THIS PARTICULAR RFA, AND THAT APPLICANTS WHO WOULD BE PUT FORWARD BY A GIVEN INSTITUTION ARE GOING TO BE MEASURED AGAINST THOSE INDIVIDUALS WHO HAVE STRONG START-UP PACKAGES, FOR EXAMPLE. SO THAT IS PART OF THE COMPETITION FOR THIS RFA.

DR. WILLIAMS: SOUNDS GOOD.

CHAIRMAN ORKIN: ONE THING I'VE ALREADY HEARD,

TALKING TO A COUPLE OF PEOPLE AT THE INSTITUTIONS, IS SOME OF THE JUNIOR FACULTY PEOPLE ARE WORRIED ABOUT WHAT HAPPENS AT THE END OF THE FIVE-YEAR PERIOD.

DR. HARI: FIVE YEARS IS A LONG TIME.

CHAIRMAN ORKIN: FROM A FOUNDATION FOR FIVE YEARS, THEY'RE ALREADY CONCERNED THAT IF THEY HAVE STRONG SUPPORT FROM CIRM AS JUNIOR FACULTY, WHEN THEY COME TO THE END OF THE FIVE YEARS, ARE THEY GOING TO BE LEFT HOLDING A LABORATORY WITHOUT MUCH FUNDS.

DR. CHIU: WE WILL LOOK AT THAT SERIOUSLY IN A COUPLE OF YEARS, BUT RIGHT NOW WE'RE JUST WORKING ON THE NEXT TWO YEARS.

CHAIRMAN ORKIN: THAT'S WHAT I TOLD THEM.

DR. CHIU: BUT IT'S A VERY LEGITIMATE QUESTION.

CHAIRMAN ORKIN: ONCE YOU GET USED TO SIRLOIN, IT'S HARD TO GO BACK TO GROUND BEEF.

MS. SAMUELSON: TO AMPLIFY ON THAT A LITTLE BIT, THEY MAY NOT BE ABLE TO GET USED TO SIRLOIN, BUT THEY SHOULD TAKE SOME COMFORT IN LOOKING AT OUR BUDGET, WHICH NOW THAT THE BONDS ARE GOING TO BE ABLE TO BE ISSUED SINCE THE LAWSUIT IS OVER, WE WILL HAVE AN INCOME STREAM OF 300 MILLION ODD A YEAR FOR SOME YEARS. AND THEY CAN BE COMPETING FOR THAT MONEY IN THE FORM OF THE ACTUAL SUBSTANTIVE GRANTS THAT WILL BE ISSUED OVER THAT TIME.

DR. CHIU: THAT'S RIGHT, JOAN. AND WE DO HAVE THE STRATEGIC PLAN, AND IT SAYS OUT YEARS, THERE ARE SHORT, MEDIUM, AND LONG-TERM INITIATIVES THAT ARE SORT OF OUTLINED THERE.

CHAIRMAN ORKIN: THEY DIDN'T GET MUCH SYMPATHY FROM ME.

I GUESS NEXT WE'RE UP TO, LET'S SEE, MAJOR FACILITIES. ARLENE, HAVE YOU --

DR. CHIU: I DON'T HAVE ANY SLIDES FOR THIS. I'M JUST GOING TO TALK QUITE QUICKLY. THIS IS ANOTHER INITIATIVE, A BIG ONE, IN CALIFORNIA THAT WAS LAUNCHED RECENTLY. AND AS YOU KNOW, ONE OF OUR GOALS IS TO PROVIDE FUNDING FOR NEW FACILITIES THAT ARE FREE OF FEDERAL FUNDING TO EXPAND THE CONDUCT OF RESEARCH AND PROVIDE FOR INTERACTION AND BUILDING OF MORE AND BETTER PROGRAMS IN CALIFORNIA.

THE FIRST SHOT AT THIS WAS THE SHARED LABS PROGRAM, AND THAT WAS SUPPOSED TO BE QUICK, BUT VERY LIMITED IN SCOPE.

THE CIRM MAJOR FACILITIES GRANT PROGRAM IS A MUCH LARGER PROGRAM TO ESTABLISH CIRM FACILITIES THAT COVERS THE FULL SPECTRUM OF RESEARCH FROM DISCOVERY TO DEVELOPMENT AND TO POSSIBLE TESTING OF CURES, THERAPIES, DIAGNOSTICS, AND TECHNOLOGIES FOR THE TREATMENT OF INJURY AND DISEASE.

THIS RFA IS ONLY OPEN TO NONPROFIT ENTITIES AND ACADEMIC ENTITIES IN CALIFORNIA, AND ALL THE PROPOSED FACILITIES MUST BE LOCATED IN CALIFORNIA. IT'S ALSO STATED IN PROP 71 THAT WE WILL BE FUNDING BUILDING OF SUCH FACILITIES.

WE'VE LIMITED APPLICATIONS, THAT EACH INSTITUTION CAN ONLY SUPPORT ONE APPLICATION, AND EACH APPLICATION IS TO ADDRESS ONE SINGLE PROJECT AT ONE SINGLE SITE.

SO NOT TO GO INTO TOO MUCH DETAIL ABOUT IT, BECAUSE WE HAVE MORE THINGS ON THE AGENDA, WE BROKE THIS APPLICATION UP, BECAUSE OF ITS COMPLEXITY, INTO TWO PARTS. THE APPLICATION PART 1 WILL DEAL WITH THE BREADTH AND DEPTH OF THE OVERALL STEM CELL RESEARCH PROGRAM AT THE APPLICANT INSTITUTION AND IDENTIFIES THE PARTS OF THE PROGRAM THAT WILL BE HOUSED IN THE PROPOSED FACILITY AND ALSO, IN GENERAL, DESCRIBE HOW IMPORTANT WILL THE NEW FACILITY BE FOR THE DEVELOPMENT OF THEIR STEM CELL PROGRAM. AND THIS WOULD BE THE SCIENTIFIC JUSTIFICATION FOR THE PROPOSED FACILITY, AND THIS WILL BE REVIEWED, THEREFORE, BY THE GRANTS WORKING GROUP FOR SCIENTIFIC MERIT.

THERE ARE NO DOLLARS ATTACHED TO THIS PART 1, BUT THERE WILL BE A LOT OF INFORMATION ON THE SCIENTIFIC PROGRAM AT EACH OF THE APPLICANT INSTITUTIONS, NOT ONLY

THAT WHICH WILL BE HOUSED IN THE PROPOSED FACILITY, BUT THE OVERALL PROGRAM AND AUXILIARY PROGRAMS AT THE INSTITUTION AND COLLABORATION, SO IT'S QUITE COMPLEX.

NOW, HAVING SUBMITTED THIS FOR YOUR REVIEW, THE CIRM -- YOUR RECOMMENDATIONS WILL BE BROUGHT TO THE CIRM GOVERNING BODY, THE ICOC, WHO CAN APPROVE ALL OR PART OF AN APPLICATION'S PART 1 APPLICATION. THE INSTITUTION WILL THEN BE INVITED TO APPLY FOR PART 2 WHERE THEY WILL ADDRESS THE TECHNICAL ASPECTS OF ITS BUILDING PROGRAM, THE COSTS INVOLVED, AND TO EXPLAIN HOW THE PROGRAM ALIGNS WITH CIRM'S OBJECTIVES. SO TWO PARTS. YOU DON'T HAVE TO WORRY ABOUT PART 2 SINCE PART 2 WILL BE REVIEWED BY THE FACILITIES WORKING GROUP, WHOSE MEMBERS ARE EXPERTS IN TECHNICAL AND CONSTRUCTION ASPECTS OF THE PROGRAM.

SO I'LL JUST CONFINE MY REMARKS TO PART 1, THE SCIENTIFIC REVIEW. EACH PROPOSAL, AS I SAID, WILL BE JUDGED ON THE BREADTH AND DEPTH OF ITS RESEARCH PROGRAM. AND WHAT DO WE MEAN BY THAT? WE'RE LOOKING AT A PROGRAM IN TWO DIMENSIONS. FOR BREADTH WE SEE THREE ELEMENTS, AND PLEASE BEAR IN MIND THE WORD "ELEMENT" MEANS SOMETHING VERY SPECIFIC. THERE IS AN X ELEMENT, A Y ELEMENT, AND A Z ELEMENT, EACH OF WHICH WILL CONSTITUTE A UP TO TEN-PAGE APPLICATION.

SO THE X ELEMENT COVERS THEIR BASIC AND DISCOVERY RESEARCH PROGRAM OR PROGRAMS. THE Y ELEMENT

WILL FOCUS ON PRECLINICAL RESEARCH WHERE BASIC DISCLOSURES AND TECHNOLOGIES ARE APPLIED TOWARD DEVELOPMENT OF TREATMENTS AND MAY INCLUDE RESEARCH SUCH AS IN VITRO ASSAYS, IN VIVO MODELS, DRUG DISCOVERY. AND FINALLY, THE Z ELEMENT INVOLVES PRECLINICAL DEVELOPMENT NOW, PRODUCT DEVELOPMENT IDEAS AND CLINICAL RESEARCH, INCLUDING PROGRAMS TO POTENTIALLY TEST OUTCOMES OF USE OF THERAPEUTICS OR PROCEDURES.

NOW, EACH APPLICANT CAN CHOOSE TO APPLY ONLY FOR THE X PART OR FOR X AND Y OR X AND Z OR Y AND Z, OR FOR ALL THREE. IT'S UP TO THEM, BUT FOR EACH PART, THEY HAVE TO WRITE A SINGLE ELEMENT PROPOSAL. AND SO THEY CAN COMPETE IN ONE, TWO, OR THREE ELEMENTS.

NOW, LET'S LOOK AT THE DEPTH OF EACH ELEMENT NOW. FOR REVIEW OF DEPTH OF A PARTICULAR PROGRAM FOR EACH OF THESE ELEMENTS, THE APPLICATION WILL BE JUDGED ON THE STRENGTHS AND WEAKNESSES OF ITS PROGRAM. AND THE DEPTH WILL BE COVERED IN JUST FOUR PARTS: THE SCIENTIFIC PROGRAM OBVIOUSLY, HOW GOOD ARE THE PI'S, HOW WELL -- HOW MUCH SYNERGISM, ETC., WILL TAKE PLACE. SECOND WILL BE ARE THERE ANY FORMAL INSTITUTIONAL COLLABORATIONS THAT THEY'VE ESTABLISHED TO BUILD UP THE STRENGTH OF THEIR PROGRAM. THE THIRD WILL BE ON THE CORE SERVICES THAT EITHER THEY ALREADY CURRENTLY HAVE OR THAT THEY PROPOSE IN THE FACILITY AND HOW WELL IT MESHES WITH OR BUILDS

UPON THE PROGRAM THAT THEY HAVE IN MIND. AND LAST, BUT NOT LEAST, THEIR PLANS FOR GROWTH OF THIS ASPECT, THIS ELEMENT OF THE PROGRAM, RECRUITMENT OF NEW FACULTY, COMMITMENTS OF ANY KINDS BY THE INSTITUTION. SO EACH ELEMENT WILL BE JUDGED ON THESE FOUR ASPECTS.

AND THEN YOU, THE MEMBERS OF THE GRANTS WORKING GROUP, WILL BE ASKED TO GIVE A SCORE FOR EACH ELEMENT AS YOU ALWAYS DO FOR EACH GRANT, BUT IT'S EACH ELEMENT. SO YOU WILL BE COMPARING X'S AGAINST X'S, Y'S AGAINST Y'S, AND Z'S AGAINST Z'S.

AFTER YOUR RECOMMENDATIONS HAVE BEEN MADE, THE ICOC WILL LOOK AT WHAT YOUR RECOMMENDATIONS ARE, SO I'LL GIVE YOU AN EXAMPLE. AN INSTITUTION MAY COME IN WITH A PROPOSAL FOR X, Y, AND Z, AND YOU GAVE THEM A SCORE OF 80 FOR X AND Y AND 20 FOR Z. THEY'RE NOT COMPETITIVE IN Z. AND THE ICOC MAY THEN DECIDE TO ACCEPT YOUR RECOMMENDATION AND JUST TELL THEM YOU'RE GOOD FOR TWO ELEMENTS, NOT THREE.

WHAT DOES THIS MEAN IF YOU ARE APPROVED OR RECOMMENDED FOR ONE ELEMENT VERSUS TWO VERSUS THREE? IT TELLS THE APPLICANT THE SIZE OF THE FACILITY THAT THEY ARE NOW INVITED TO APPLY FOR, AND THERE ARE DIFFERENT DOLLAR AMOUNTS ASSOCIATED WITH A ONE-ELEMENT, A TWO-ELEMENT, OR A THREE-ELEMENT PROGRAM. I THINK WE HAVE NAMES LIKE CIRM INSTITUTE WOULD BE A THREE-ELEMENT

PROGRAM, CIRM CENTER FOR EXCELLENCE WOULD BE A TWO-ELEMENT PROGRAM, AND CIRM SPECIAL PROGRAM WOULD BE A ONE-ELEMENT PROGRAM.

SO THIS WAY THE SCIENCE WILL DETERMINE THE RANGE OF A PROGRAM OR PROGRAMS THAT AN APPLICANT IS SOLID OR HIGHLY RECOMMENDED FOR. THE ICOC WILL THEN TELL THEM YOU MAY NOW COME IN WITH AN APPLICATION AND PLANS FOR THAT PARTICULAR SIZE OF PROGRAM WITH DOLLAR AMOUNTS BRACKETS ATTACHED. AND THAT WILL GO TO THE FACILITIES WORKING GROUP TO SEE HOW GOOD ARE THEIR PLANS, HOW WELL THEY MEET THE ELEMENTS OF GETTING IT DONE QUICKLY, URGENCY, APPROPRIATE OBJECTIVES OF CIRM IN TERMS OF CO-FUNDING, LEVERAGE, AND MANY OTHER CONSIDERATIONS. BUT IT WOULD NOT BE THE GRANTS WORKING GROUP THAT WILL BE WORRIED ABOUT THAT ASPECT OF IT.

AND THEN IN THE END, THE ICOC, AGAIN, WILL LOOK AT THE RECOMMENDATIONS COMING FROM THE FACILITIES WORKING GROUP AND DECIDE -- DETERMINE HOW MUCH FUNDING WILL GO TO EACH OF THESE PROPOSALS.

THAT IN A NUTSHELL IS THE FACILITIES PROGRAM WHICH IS POSTED ON THE WEB, AT LEAST PART 1 IS PRETTY CLEARLY DEFINED. PART 2 IS ALSO DEFINED, BUT MORE INSTRUCTIONS WILL COME AS WE SEE WHO GETS INVITED. ARE THERE ANY QUESTIONS?

DR. BRIVANLOU: ARLENE, THIS IS ALI BRIVANLOU

AGAIN AT THE ROCKEFELLER. I ASSUME THAT THE PART OF THE BUDGET FOR MAKING NEW BUILDINGS AND NEW SPACE AVAILABLE WOULD BE SOLELY DEDICATED TO THE HUMAN PART AS THE NONHUMAN PARTS DO NOT REALLY NEED ANY PRIVATE FACILITIES.

DR. CHIU: CAN YOU SAY THAT AGAIN, ALI? ARE SOLELY ASSIGNED TO WHICH PARTS, TO THE HUMAN VERSUS NONHUMAN?

ACTUALLY WE'RE BEING BROADER THAN THAT, BUT YOU WILL JUDGE WHEN THEY COME IN ABOUT THEIR WHOLE STEM CELL PROGRAM. THIS IS NOT JUST HUMAN ONLY, BUT HUMAN AND NONHUMAN. WE WANT TO INTEGRATE THESE FOR THE PEOPLE WHO, NOT JUST IN HUMAN OR NONHUMAN STEM CELLS, BUT IMAGING, CORE FACILITIES, MAYBE EVEN GMP. I DON'T KNOW WHAT'S GOING TO COME IN. I'M JUST THINKING OFF THE TOP OF MY HEAD. HIGH THROUGHPUT, WHATEVER THEY THINK WOULD FACILITATE THEIR WHOLE STEM CELL PROGRAM. IN PARTICULAR, OF COURSE, THE HUMAN EMBRYONIC STEM CELL PROGRAM WILL COME TO YOU FOR YOUR COMMENTS AND EVALUATIONS. WE DON'T KNOW WHAT'S GOING TO HAPPEN YET.

THE LETTERS OF INTENT DON'T COME IN UNTIL SEPTEMBER 24TH, AND THE APPLICATIONS ARE DUE OCTOBER 16TH FOR PART 1. SO WE'RE NOT SURE WHAT'S GOING TO COME IN YET, BUT THERE'S A LOT OF INTEREST OUT THERE.

MR. KLEIN: ARLENE, IT MIGHT BE HELPFUL IF EVERYONE UNDERSTOOD THE SCALE HERE. THE RFA IS FOR 227,

$230 MILLION. AND AS A MEMBER OF THE FACILITIES GROUP, WHICH I'M ALSO A MEMBER WITH JEFF SHEEHY AND JANET WRIGHT AND JOAN SAMUELSON, WHO I KNOW ARE ON THIS CALL, THERE MAY BE OTHER MEMBERS OF THAT WORKING GROUP ON THE CALL AS WELL. WITHIN THE SECOND PART OF THIS, PART OF WHAT THEY'LL BE JUDGED UPON IS LEVERAGE. IF THE INSTITUTION IS MAKING A SUFFICIENT COMMITMENT TO REALLY GET THE MAXIMUM LEVERAGE FOR STEM CELL RESEARCH IN CALIFORNIA, AND I, SPEAKING AS AN INDIVIDUAL, WOULD HOPE TO SEE AT LEAST A ONE-TO-ONE LEVERAGE, WHICH WOULD MEAN THAT WE'RE IN A $450 MILLION RANGE ON FACILITIES. AND I WOULD ACTUALLY HOPE AND BELIEVE FROM PUBLIC ANNOUNCEMENTS OF PROJECTS UNDER WAY THAT WE'RE AWARE OF IN THE STATE THAT WE WILL HAVE OVER $500 MILLION OF FACILITIES HERE.

SO THOSE FACILITIES MIGHT INCLUDE VIVARIUM, MIGHT NOT, MIGHT INCLUDE OTHER SPECIALIZED FACILITIES, HIGH THROUGHPUT FACILITIES OR SPECIAL AREAS DEDICATED TO VERY HIGH THROUGHPUT SCREENING ON A VERY LARGE-SCALE WITH ROBOTICS, FOR EXAMPLE.

WHAT'S IMPORTANT HERE IS TO REALIZE THAT THIS FIRST PART THAT ARLENE HAS DESCRIBED, THE SCIENCE IS REALLY DRIVING THIS. AND SCIENTIFIC SCORES, AS ARLENE INDICATED, ARE GOING TO BE THE MAJOR INSTRUCTION TO THE BOARD ON HOW TO CLASSIFY AND PROVIDE INVITATIONS FOR THE SECOND PART OF THE PROCESS, WHICH WILL RESULT IN THE

AWARD.

DR. CSETE: I HAVE SIMILAR CONCERNS TO ALI; BUT IN ADDITION, UNDERSTANDING THE VALUE OF COMPETITION BETWEEN INSTITUTIONS IN CALIFORNIA, I'M WONDERING, SINCE THE SCALE OF THESE IS SO LARGE, WHETHER THERE'S INCENTIVE FOR PEOPLE TO OPEN THEIR FACILITY TO OTHER INSTITUTIONS WITHIN CALIFORNIA. SO, FOR EXAMPLE, HOW MANY GMP FACILITIES DO YOU REALLY NEED? AND SINCE THIS IS A STATE INITIATIVE, WHETHER THAT SHOULD ALSO BE PART OF THE CONSIDERATION, HOW THEY DRIVE THE OVERALL STATE EFFORT WITH THIS NEW FACILITY THAT THEY'RE PROPOSING.

DR. CHIU: COLLABORATION IS ONE OF THE KEY ELEMENTS. WE ASK FOR BOTH FORMAL PARTNERSHIPS, AND I HEAR THAT THERE ARE ALL KINDS OF LESS FORMAL. A RANGE OF PARTNERSHIPS HAVE BEEN PLANNED. SO I THINK YOU WILL FIND THAT THERE WILL BE A LOT OF THAT IN THE APPLICATIONS.

MR. KLEIN: IN THE SECOND PART OF THE EVALUATION, THERE'S AN ACTUAL POINT CATEGORY FOR SOMETHING CALLED SHARED RESOURCES. SO THAT, FOR EXAMPLE, IF ONE INSTITUTION IS BUILDING A MAJOR FACILITY, BUT THEY CAN'T AFFORD TO PUT A VIVARIUM IN IT AND THEY HAVE A COLLABORATION OR A FORMAL WRITTEN PARTNERSHIP WITH ASSURED ACCESS TO A VIVARIUM AT ANOTHER INSTITUTION, THEY GET POINTS FOR THIS COLLABORATION BECAUSE IT ESSENTIALLY SAVES MONEY TO THE STATE AS A PROGRAM NOT TO COMPLETELY

DUPLICATE.

AND GMP IS ANOTHER ACTUAL EXAMPLE THAT YOU JUST BROUGHT UP, THAT IF THERE'S SOMEONE WITH A GMP FACILITY THAT HAS A FORM OF COLLABORATION WITH SOMEONE WHO IS MAKING THE GRANT APPLICATION, THE GRANT APPLICATION WILL GET POINTS FOR NOT DUPLICATING A GMP FACILITY WHEN THEY HAVE ACCESS THROUGH THIS FORMAL PARTNERSHIP TO THAT OPPORTUNITY.

CHAIRMAN ORKIN: ANY OTHER COMMENTS? GOOD. THANK YOU, ARLENE. SOUNDS VERY INTERESTING.

THE NEXT TOPIC IS THAT OF DISEASE TEAM INITIATIVE, A NEW REPORT, AND BETTINA STEFFEN WILL BE DISCUSSING THIS.

DR. STEFFEN: I UNDERSTAND THE WEBCAST MIGHT NOT BE WORKING FOR ALL OF YOU, SO I'LL TRY TO CUE YOU INTO WHICH OF THE SLIDES WE'RE LOOKING AT.

AND JUST AS A STARTING POINT, I'D LIKE TO TELL YOU WHERE CIRM IS IN THE PROCESS OF DEVELOPING THIS INITIATIVE. RIGHT NOW WE ARE STILL AT THE CONCEPT DEVELOPMENT STAGE, SO THIS IS AN INFORMATION GATHERING MODE.

AND THE SECOND REASON THAT WE SHOULD SPEND SOME TIME ON THIS TODAY IS THAT THIS WILL BE A DEPARTURE FROM THE TRADITIONAL RESEARCH GRANTS. WE'LL SEE TEAM-BASED RESEARCH, MORE ACTIVE MANAGEMENT ON THE PART OF THE

AGENCY, AND SO WE THOUGHT IT WOULD BE WORTHWHILE TO TAKE TIME TO BRIEF YOU ON THE INITIATIVE AND ASK FOR YOUR INPUT.

I'VE MOVED ON TO THE AGENDA SLIDE. WE'LL TALK BRIEFLY ABOUT THE INITIATIVE AND THEN GO THROUGH THE KEY POINTS THAT CAME OUT IN OUR WORKSHOP AND THEN OPEN THIS UP TO DISCUSSION WITH YOU.

WE'RE NOW LOOKING AT THE DISEASE TEAM INTENT SLIDE. CIRM HAS A SCIENTIFIC STRATEGIC PLAN WHICH WAS ADOPTED IN 2006 THAT ESTABLISHED A BLUEPRINT FOR ACHIEVING THE INSTITUTE'S GOALS. AND SEVERAL FUNDING PROGRAMS ARE DESCRIBED IN THERE, EACH TARGETING DIFFERENT AREAS OF RESEARCH. AMONG THEM IS THE DISEASE TEAM INITIATIVE.

AND THE INTENT OF THE INITIATIVE IS "TO EXPLORE A NEW METHOD OF INTEGRATING AND ORGANIZING THE HIGHEST QUALITY BASIC, TRANSLATIONAL, AND CLINICAL RESEARCH WITH A SPECIFIC AIM OF PRODUCING A THERAPY FOR A PARTICULAR DISEASE OR GROUP OF DISEASES WHOSE RESEARCH IS POISED FOR THE DEVELOPMENT OF THERAPIES."

MOVING ON TO THE RATIONALE SLIDE, THE REASON THAT DISEASE TEAMS ARE IMPORTANT IS THAT DEVELOPMENT CAN PROCEED FASTER AND MORE EFFECTIVELY IF THREE CONDITIONS ARE MET. THE MULTIDISCIPLINARY MEMBERS SHOULD PARTICIPATE IN ALL PHASES OF THE PROJECT. AND THIS

ENCOURAGES EARLY CONSULTATION AND COOPERATION WITH RESEARCHERS OF DIVERSE EXPERTISE. AND WITH THIS INITIATIVE, WE WILL ALSO BE TESTING WHETHER EMPHASIZING A COMPREHENSIVE RESEARCH PLAN, COMPLETE WITH MILESTONES AND ACTIVE TEAM MANAGEMENT, CAN RESULT IN A MORE EFFICIENT PROCESS.

YOU SHOULD NOW BE LOOKING AT THE DISEASE TEAM INITIATIVE CONCEPT, AND THERE ARE THREE BIG OVALS AT THE TOP OF THE SLIDE. THE INITIATIVE HAS THREE PARTS: A WORKSHOP, WHICH WAS HELD IN JULY OF 2007; A PLANNING GRANT, WHICH WE ANTICIPATE IN THE FALL OF 2007; AND A DISEASE TEAM GRANT ANTICIPATED IN THE SUMMER OF 2008.

AND THE DATES IN ORANGE OR THE ONES THAT ARE ASTERISKED, IF YOU'VE PRINTED THIS OUT, ARE JUST TENTATIVE DATES. THEY ARE ANTICIPATED DATES.

THE PURPOSE OF THE WORKSHOP WAS TO ASSEMBLE RESEARCHERS AND FUNDING AGENCIES WHO ALL HAD EXPERIENCE WITH TEAM-BASED PROJECTS AND HAVE THEM TELL US WHAT WORKS IN THOSE SETTINGS. WE ASKED ABOUT THE SCOPE, RESOURCES, PROJECT MANAGEMENT, AND FUNDING OF EFFECTIVE TEAMS, AND THE STRENGTHS AND WEAKNESSES OF THOSE APPROACHES. WE ALSO ASKED STEM CELL SCIENTISTS TO IDENTIFY REQUIREMENTS UNIQUE TO THIS TYPE OF RESEARCH.

THE PURPOSE OF THE PLANNING GRANT IS TO ALLOW POTENTIAL TEAMS TO ASSEMBLE AND TO PREPARE A

COMPREHENSIVE RESEARCH PROPOSAL.

AND, FINALLY, IN THE SUMMER OF 2008, WE ANTICIPATE THE LARGE DISEASE TEAM GRANTS, WHICH WILL BE MULTIYEAR EFFORTS.

I'VE MOVED ON TO THE DISEASE TEAMWORK SHOP SLIDE. IT WAS HELD IN JULY 2007 ON THE 25TH AND 26TH IN SAN FRANCISCO, AND OUR PARTICIPANTS INCLUDED A WEALTH OF EXPERTISE. WE HAD SCIENTISTS FROM ACADEMIA AND INDUSTRY, WE HAD PATIENT ADVOCATE REPRESENTATIVES, REPRESENTATIVES FROM FEDERAL FUNDING AND REGULATORY AGENCIES, AND ALSO PRIVATE FOUNDATIONS WHICH FUND DISEASE-TARGETED RESEARCH. CIRM STAFF ALSO PARTICIPATED IN THE SESSION.

ON THE NEXT PAGE, WHICH SHOULD LOOK LIKE A LIST OF NAMES IN BOXES, IN THE TOP BOX ARE SCIENTISTS WHO PARTICIPATED IN THE SESSION. AND ON THE LEFT THERE ARE SIX ACADEMIC PARTICIPANTS AND FIVE WHO CAME FROM VARIOUS CORPORATIONS. IN THE MIDDLE ROW ON THE LEFT, WE HAVE OUR TWO PATIENT ADVOCATES WHO ARE ALSO ICOC BOARD MEMBERS AND ALSO SERVE ON THE GRANTS WORKING GROUP. THANK YOU, JOAN SAMUELSON AND JEFF SHEEHY, FOR PARTICIPATING WITH US.

WE HAD TWO PARTICIPANTS FROM THE FDA AND FOUR PARTICIPANTS FROM FUNDING AGENCIES, ON THE RIGHT FROM THE NIH, AND ON THE LEFT ARE THREE PARTICIPANTS FROM PRIVATE FOUNDATIONS, AGAIN WHO ALL FUND DISEASE-SPECIFIC RESEARCH.

THE WORKSHOP WAS HELD OVER TWO DAYS, AND ON THE FIRST DAY WE ASKED PARTICIPANTS WHO HAD WORKED WITH TEAMS TO GIVE A PRESENTATION OF THEIR TEAM MODELS AND TO TELL US WHAT WORKS AND, FRANKLY, WHAT DOESN'T. ON THE SECOND DAY WE ASSEMBLED TO DISCUSS SPECIFIC TOPICS LISTED HERE. WE ADDRESSED THE SCOPE OF DISEASE TEAMS, THE ORGANIZATION OF DISEASE TEAMS, ACTIVE MANAGEMENT AND OVERSIGHT, RESOURCES THAT MIGHT BE UNIQUE TO TEAMS, AND OPERATIONAL CHALLENGES.

ON THE NEXT FEW SLIDES, WE'LL GO INTO THE KEY FINDINGS FROM THESE SESSIONS. WE'RE NOW LOOKING AT PROPOSED SCOPE OF DISEASE TEAMS, AND THIS IS THE SERIES OF ARROWS ON YOUR SLIDE. THIS DIAGRAM REPRESENTS THE PATH THAT A TYPICAL THERAPY WOULD TAKE FROM DISCOVERY TOWARD CLINICAL TESTING. IN OUR WORKSHOP WE WERE FOCUSED ON THE BLUE SHADED AREAS FROM LATE DISCOVERY THROUGH PRECLINICAL RESEARCH AND UP TO INCLUDING SOME CLINICAL ACTIVITIES.

WE HEARD MUCH DISCUSSION COMPARING RESEARCH AT THE TWO ENDS OF THIS SPECTRUM. FOR EXAMPLE, EARLY PROJECTS IN DISCOVERY HAVE VERY FLUID TASKS, AND MILESTONES ARE DISCOVERY DRIVEN, AND A CLEAR PATH MAY NOT BE WELL DEFINED. AT THE RIGHT END OF THE SPECTRUM, AS PROJECTS APPROACH IND FILING AND CLINICAL TESTING, TASKS AND MILESTONES ARE PREDICTABLE AND ARE LARGELY DRIVEN BY

REGULATORY REQUIREMENTS.

WHAT WE HEARD CRYSTALIZING FROM THE DISCUSSION IS THAT THE IDEAL PROJECTS FOR DISEASE TEAMS WOULD BE TRANSLATIONAL EFFORTS WITH AN END POINT OF FILING AN APPROVABLE IND AND ENTRY INTO CLINICAL TRIALS. AND THIS LESSON CAME TO US FROM THE NATIONAL CANCER INSTITUTE'S TRANSLATIONAL EFFORTS. THE EXAMPLE THAT WAS CITED THERE IS IF YOU NAME THE END POINT AS FILING AN IND, YOU MIGHT GET A STACK OF A HUNDRED IND'S OUT OF THE PROGRAM AND RELATIVELY FEW OR NO PROJECTS IN CLINICAL TRIALS. BUT IF YOU CAREFULLY ARTICULATE THE END POINT AS REQUIRING AN APPROVED IND AND ENTRY INTO THE CLINICAL TRIALS, THEY HAD A MUCH HIGHER SUCCESS RATE OF PROJECTS ACTUALLY ENTERING THE CLINICS. SO WE WILL BE CAREFUL ABOUT HOW WE NAME THAT END POINT FOR THE PROGRAM.

PROJECTS MIGHT START ANYWHERE UPSTREAM IN THAT BLUE BAR, BUT WE THINK THAT A REASONABLE MINIMAL CRITERIA FOR THESE PROJECTS WOULD BE INITIAL EVIDENCE OF DISEASE MODIFYING ACTIVITY.

I KNOW THAT'S A LOT TO DIGEST, SO I WONDER IF THIS IS AN APPROPRIATE PLACE TO PAUSE FOR QUESTIONS OR COMMENTS.

DR. CSETE: I WANT TO WAIT TILL THE END. ONE OF THE THINGS I WAS CONCERNED WITH WHEN I LOOKED THROUGH THIS THE FIRST TIME, AND MAYBE CALIFORNIA IS JUST WAY

AHEAD ON THIS, EVEN WITH AUTOLOGOUS STEM CELL THERAPIES FOR UNUSUAL APPLICATIONS, I THINK ONE OF THE THINGS THAT STOPS PEOPLE ON THE GROUND RIGHT NOW IS THAT IRB'S ARE NOT FUNDED. I'M WONDERING IF PART OF -- THERE SHOULD BE SOME EXPLICIT ADDRESSING OR EDUCATION OF IRB'S AS PART OF THIS JUST SO YOU DON'T GET TO A POINT WHERE THAT'S AN OBSTRUCTION ON THIS ROAD MAP.

DR. STEFFEN: THAT'S AN EXCELLENT POINT, AND I THINK WE HEARD THAT ECHOED IN THE WORKSHOP MANY TIMES.

DR. BRIVANLOU: I ALSO WOULD LIKE TO SECOND WHAT WE JUST HEARD BECAUSE MY EXPERIENCE ALSO IN THE NORTHEAST, THAT THE LIMITING FACTORS ARE USUALLY AT THE IRB LEVEL, NOT THE COMMITTEES.

MR. KLEIN: COULD THAT POINT BE EXPANDED A LITTLE MORE FOR THE BENEFIT OF SOME OF US WHO MAY NOT HAVE HEARD AS MUCH INFORMATION ON THE IRB'S AS LIMITING FACTORS?

DR. CSETE: I CAN JUST TELL YOU LOCAL EXPERIENCE. AND I THINK WE HAVE TO INCLUDE IACUC IN THIS A LITTLE BIT AS WELL. SO IF YOU HAVE AN IDEA THAT YOU CAN EVEN GET FUNDING FOR FROM ATYPICAL SOURCES LIKE THE DEFENSE DEPARTMENT, FOR EXAMPLE, TO USE AUTOLOGOUS CELLS, SO A DO NO HARM KIND OF APPROACH FOR UNUSUAL APPLICATIONS, YOU CAN'T ENROLL PATIENTS UNTIL YOU HAVE AN APPROVED IRB PROTOCOL OBVIOUSLY. AND THERE IS

CONSIDERABLE VARIATION FROM INSTITUTION TO INSTITUTION, AND REALLY FROM YEAR TO YEAR, I THINK, BECAUSE OF THE FLUIDITY OF SOME OF THESE COMMITTEES ABOUT WHAT IS -- WHAT THEY WILL ALLOW IN AN INSTITUTION.

AND I THINK A LOT OF THE IRB'S JUST SORT OF DRAW A LINE IN THE SAND AND SAY WE'RE NOT GOING TO USE STEM CELLS EXCEPT FOR APPROVED HEMATOPOETIC STEM CELL TRANSPLANT KINDS OF THERAPIES BECAUSE THEY ARE UNEDUCATED AS TO WHAT EXACTLY THE PARTICULAR CELL IS AND WHAT EXACTLY ITS POTENTIAL IS. AND THEY JUST DON'T WANT TO GET INTO TROUBLE EITHER AT THE LEVEL OF FEDERAL REGULATIONS. I THINK THAT DOESN'T -- THAT WON'T HAPPEN BECAUSE NO ONE IS GOING TO PUT HUMAN EMBRYONIC STEM CELLS IN CALIFORNIA. BUT ALSO AT THE LEVEL OF JUST THINKING ABOUT PATIENT SUITS AND THINGS LIKE THIS AS PATIENTS ARE ENROLLED IN PROTOCOLS.

SO THERE'S AN ALMOST AUTOMATIC RESPONSE TO SAY NO TO INVESTIGATORS, I THINK, AT SOME INSTITUTIONS IF THEY'RE REALLY TRYING TO DO SOMETHING NEW CLINICALLY EVEN THOUGH IT'S CLINICALLY VERY WELL THOUGHT OUT. AND I'M BEING THE OPTIMIST WHEN I SAY THAT I THINK EDUCATION OF IRB'S REALLY CHANGES THIS, BUT THAT'S THE ONLY WAY THAT WE CAN REALLY APPROACH IT.

MR. KLEIN: OS STEWARD IS THERE; IS THAT RIGHT?

DR. STEWARD: YES.

MR. KLEIN: WOULD IT BE APPROPRIATE IN TERMS OF THE AGENDA OF THE PRESENTER OR ARLENE, YOUR VIEW, TO HAVE OS JUST COMMENT GIVEN THAT HIS INSTITUTION AT UC IRVINE HAS GONE THROUGH THIS PROCESS?

DR. CHIU: WE'D LOVE TO HEAR DR. STEWARD IF HE HAS ANY INSIGHT TO BRING TO THIS.

DR. STEWARD: SO CAN YOU HEAR ME? SO I GUESS, BOB, YOU'RE REFERRING TO THE -- SOME OF THE PROTOCOLS THAT ARE UNDER WAY THAT DON'T INVOLVE HUMAN EMBRYONIC STEM CELLS. WE'RE NOT YET INVOLVED IN ANY OF THE HUMAN EMBRYONIC STEM CELL CLINICAL TRIALS.

AND I THINK WHAT'S BEEN SAID IS REALLY COMPLETELY ON THE MARK. I THINK EVERYONE NEEDS TO UNDERSTAND THAT IRB'S ARE LOCALLY RUN AT THE INSTITUTIONAL LEVEL, AND THERE'S A VERY STRONG COMMITMENT TO THAT LOCAL CONTROL. SO I TOTALLY AGREE THAT IT IS LIKELY TO BE AN ISSUE DOWN THE ROAD. I DON'T KNOW THAT CIRM CAN REALLY DO ANYTHING ABOUT THAT EXCEPT PLAY MAYBE A CHEERLEADER ROLE.

DR. CHIU: I THINK YOU BRING UP A POINT THAT WE HAD NOT APPRECIATED BEFORE, THAT THE IRB'S MIGHT BE, IF NOT A LIMITING FACTOR, CERTAINLY COULD BE A CHALLENGE TO SOME OF THESE APPLICANTS. AND IT OCCURRED TO ME THAT STEM CELL, INC., FOR EXAMPLE, IS ALREADY INTO A PHASE I TRIAL USING THEIR CELLS. AND PERHAPS WE MIGHT THINK

ABOUT GATHERING A FEW EXPERT OPINIONS ON HOW TO GET THROUGH THIS.

IF, SAY, MAYBE JUST EVEN BEGINNING STEPS OF IF WE SHOULD FUND A DISEASE TEAM, THAT WITHIN TWO YEARS OR SO BEFORE THEY'RE READY TO LAUNCH, BEGIN TO WORK WITH IRB'S TO GET THEM UP TO SPEED TO HELP THEM THROUGH. THAT'S A VERY CRITICAL POINT THAT COULD HALT PROGRESS.

DR. CSETE: A MORE STABLE PERSON IN MEDICAL SCHOOLS, ANYWAY, ARE THE PEOPLE WHO HEAD UP THE COMPLIANCE OFFICE. AND I THINK IF YOU ARE GOING TO BE WORKING WITH IRB'S, YOU SHOULD BE WORKING WITH THE COMPLIANCE OFFICERS AS WELL.

DR. CHIU: GOOD TO KNOW.

DR. BRIVANLOU: THE SECOND ISSUE THAT WAS BROUGHT UP ON THE IRB WAS THE IACUC ISSUE. FOR THOSE OF YOU WHO DON'T KNOW WHAT IACUC IS, THIS IS A GROUP OF PEOPLE DOWNSTREAM OF ALL THE APPROVALS AFTER YOU ACTUALLY PASS THE COMPETITION, GET THE GRANT, AND YOU HAVE THE PRIVATE SPACE TO WORK ON, AND YOU HAVE THE POST-DOCS READY TO DO THE WORK, AND YOU PASS YOUR IRB. THEN THE IACUC, WHICH DEALS WITH THE SAFETY AND HEALTH OF ANIMALS THAT ARE USED IN EXPERIMENTS.

SO ONE OF THE MOST LIMITING FACTORS FROM OUR SIDE HERE IN NEW YORK AND ALSO IN BOSTON, I CAN TELL YOU, IS NOT THE FACT THAT LIMITATION HAS BEEN REALLY AT THE

LEVEL OF IRB COMMITTEES, BUT SOME COLLEAGUES, SOME SCIENTISTS, SOME PEOPLE THAT I RESPECT VERY, VERY MUCH, HAVE A DIFFICULT TIME TO ACCEPT IN VIVO EXPERIMENTS THAT MIGHT BE REQUIRED TO PURSUE THE FIELD AT THE BASIC RESEARCH ON THE SAME STANDARD AS WHAT HAS BEEN USED AND WE'VE BEEN USING FOR LONG, LONG TIME IN MODEL SYSTEMS.

THIS IS NOT SOMETHING THAT ADMINISTRATIVELY CAN BE SOLVED UNFORTUNATELY. AND I THINK IT CONSTITUTES ONE OF THE MOST BASIC ELEMENTS OF WHAT I WAS TALKING ABOUT BEFORE ABOUT SELF-CENSORSHIP. PEOPLE DON'T COME BECAUSE THEY'RE NOT INTERESTED OR THEY DON'T HAVE THE TALENT. THE REAL IMPETUS IS SO MANY HURDLES THAT THEY HAVE TO OVERCOME, THAT IT MAKES THEM (UNINTELLIGIBLE).

DR. CSETE: I WAS ABOUT TO GO TO IACUC, ALI, SO I REALLY APPRECIATE THAT YOU ALWAYS ARE MUCH MORE ELEGANT IN YOUR SPEECH THAN I AM, BUT I THINK IT'S A BIG PROBLEM.

MR. KLEIN: AND HOPEFULLY, OS, THROUGH HANS KEIRSTEAD AND HIS INTERFACE WITH GERON, YOU COULD PROVIDE INSTITUTIONALLY THROUGH YOUR TEAMS THERE THAT ARE WORKING WITH GERON SOME INSIGHTS THAT WOULD BE HELPFUL.

DR. STEWARD: WELL, I WOULD JUST HAVE TO SAY, BOB, THAT AT THE MOMENT UC IRVINE IS NOT ONE OF THE SITES THAT IS BEING -- THAT IS BEING PROPOSED AS A SITE FOR GERON'S CLINICAL TRIALS. SO, IN FACT, WE'RE NOT GOING TO BE DIRECTLY INVOLVED IN THAT AT THE MOMENT.

IF I COULD JUST SAY SOMETHING, THOUGH, ABOUT THE IACUC. THE ONE QUESTION ABOUT CALIFORNIA THAT MAYBE WE ARE A LITTLE BIT AHEAD IS THAT THERE ARE DISCUSSIONS UNDERWAY AT THE IACUC LEVEL AND ALSO OCCASIONALLY AT THE IRB LEVEL OF LET'S CALL THEM SORT OF DOWNLOADABLE PROTOCOLS THAT PEOPLE, AND I'LL JUST GIVE YOU AN EXAMPLE, MAYBE DOING SPINAL CORD INJURY RESEARCH IN RODENTS, TRANSPLANTING CELLS IN, RATHER THAN JUST STARTING ALL OVER AGAIN WHEN YOU WRITE YOUR IACUC PROTOCOL, HAVING A UC SYSTEMWIDE DOWNLOADABLE FORM THAT SORT OF FILLED IN ALL THE CRITICAL INFORMATION.

STILL THE CONTROL WOULD BE AT THE LOCAL INSTITUTIONAL LEVEL, BUT AT LEAST YOU WOULD THEN BE COMFORTABLE THAT SOME OTHER INSTITUTION HAD SIGNED OFF ON THIS.

THERE IS DISCUSSION ABOUT DOING THAT AT THE IRB LEVEL AS WELL, ALTHOUGH I THINK THAT'S NOT AS FAR ALONG AS THE IACUC LEVEL BECAUSE THEY'RE SORT OF TRADITIONALLY MUCH MORE, I THINK, FEELING OF THE NEED FOR LOCAL CONTROL AT THE IRB LEVEL.

HAVING SAID THAT, WE'RE STILL ONLY TALKING ABOUT UC SYSTEMS, BUT I THINK IF THAT BEHEMOTH ACTUALLY GETS GOING, THEN THE OTHER INSTITUTIONS MIGHT WANT TO PLAY ALONG. AGAIN, I THINK CIRM MIGHT BE ABLE TO HELP IN THAT REGARD AS SORT OF A REPOSITORY OF SOME OF THIS

INFORMATION IF IT WAS POSSIBLE TO DO.

DR. CHIU: YES. I HAD NOT THOUGHT ABOUT THIS, BUT I THINK THAT'S A GREAT POINT. AND BETTINA POINTED OUT TO ME THAT THAT ISSUE, IN GENERAL, WAS RAISED ABOUT PROVIDING INFORMATION TO IACUC, TO IRB MEMBERS, TO REGULATORY ELEMENTS, LOCAL REGULATORY ELEMENTS, SO THAT THEY'RE ON THE SAME PAGE. AND I THINK IF THE UC SYSTEM HAS CERTAIN STANDARD FORMS OR TEMPLATES, AND IF THERE WAS AN OCCASION FOR THESE GROUPS TO COME TOGETHER ONCE A YEAR OR SOMETHING AND SHARE EXPERIENCES AND STANDARDS, MAYBE THAT WOULD HELP THIS FORWARD OR AT LEAST SMOOTH THE PATH FOR IT AND MAKE IT MORE CONSISTENT.

I DON'T KNOW IF THAT WOULD WORK OR NOT. IF THE GRANTS WORKING GROUP MEMBERS IN THEIR PERSONAL EXPERIENCE HAVE OTHER SUGGESTIONS TO GET OVER THESE REGULATIONS OR REGULATORY CONSIDERATIONS, WE WOULD REALLY APPRECIATE HEARING THEM EARLY.

DR. CSETE: SORT OF ALONG THE SAME LINES, ONE OF THE THINGS THAT REALLY DISTURBS ME IN THE PROCESS OF GOING FROM BENCH TO ANIMAL EXPERIMENT TO HUMAN IS KIND OF AN INTRANSIGENCE ON THE PART OF IACUC'S AND VETERANARIANS THAT WORK WITH THEM, THAT THERE'S ANIMAL-SPECIFIC ANESTHETICS, FOR EXAMPLE. HERE WE ARE PLANNING TO PUT X CELLS INTO A HUMAN FOR A THERAPY, AND OUR ANIMAL MODELS ARE TREATED COMPLETELY DIFFERENTLY IN THE PROTOCOL

BECAUSE OF THE WAY IACUC'S ARE INFLUENCED BY THE VETERINARIANS THAT CONTROL THEIR REGULATIONS.

SO I JUST THINK THAT PART OF THIS EDUCATIONAL PROCESS IS MOVING, YOU KNOW, LONG HISTORIES OF THE WAY ANIMALS ARE HANDLED IN ORDER TO VIRTUALLY REPRESENT WHAT'S GOING TO HAPPEN TO A PATIENT WHO'S UNDERGOING THE SAME THERAPY. AND IF WE DON'T EMPHASIZE THAT AS PART OF THE RFA, IT'S NOT GOING TO CHANGE.

DR. CHIU: THAT'S A THOUGHT.

DR. BONNER-WEIR: I WANTED TO ASK, LISTENING TO THAT, IN THAT I AM ON OUR IACUC HERE, AND WHEN WE HAVE A VERY DIFFICULT ISSUE ABOUT A PROTOCOL THAT SOMEBODY IS WANTING, WHAT HELPS US IS TO KNOW WHAT OTHER INSTITUTIONS HAVE DONE. SO I THINK WHAT YOU WERE SAYING, THAT IF THERE'S SORT OF A UC-WIDE THING THAT ACTUALLY DOES GO THROUGH, THEN THAT ACTUALLY, AS LONG AS IT'S MADE AVAILABLE AT A MEETING, MAYBE NOT, BUT AT LEAST ALL THE STEM CELL FUNDED PEOPLE FROM CIRM CAN BE ALERTED THERE ARE THESE TEMPLATES AVAILABLE THAT HAVE GONE THROUGH AND HAVE BEEN VETTED BY THE UC SYSTEM ALREADY THAT MAY HELP YOU IN THIS REGARD.

THAT WOULD BE -- THAT WOULD BE AN EFFECTIVE WAY TO GET SOME OF THE INFORMATION OUT.

DR. CHIU: WE COULD CERTAINLY TRY TO HELP THAT PROCESS ALONG ON A PARALLEL TRACK.

DR. MURPHY: BETTINA, THIS NOTION OF INITIAL EVIDENCE OF DISEASE MODIFYING ACTIVITY, OBVIOUSLY WE ALL KNOW WHY THAT'S THERE. BUT WHAT LOOKS PROMISING TODAY TOMORROW MAY BE A FALSE POSITIVE OR IT JUST MAY NOT WORK OUT; AND, THEREFORE, BY THAT TIME YOU MAY HAVE A GROUP THAT'S ALREADY FUNDED AND TOGETHER, WHICH MAY BE PRODUCTIVE OR MAY NOT BE PRODUCTIVE.

THE OTHER WAY OF LOOKING AT THIS WOULD BE TO TAKE THE POWER OF A MULTIDISCIPLINARY TEAM FROM BASIC TO CLINICAL AND HAVE THEM IDENTIFY IN A DISEASE OR IN A FIELD WHAT THE BOTTLENECKS IN THAT FIELD ARE. AND ALTHOUGH THEY MAY NOT HAVE ANYTHING THAT AS YET IS DISEASE MODIFYING ACTIVITY, BRINGING THESE PEOPLE TOGETHER WITH INNOVATIVE APPROACHES TO IDENTIFYING THE BOTTLENECKS AND COMING UP WITH STRATEGIES TO BREAK THROUGH THE BOTTLENECKS MAY ALSO BE VALUABLE.

I'M WONDERING IN THE COURSE OF THE WORKSHOPS, DID YOU GO EARLIER THAN DISEASE MODIFYING ACTIVITY, AND WHAT WAS THE FEELING OF THE COMMITTEE?

DR. STEFFEN: WE CERTAINLY HEARD THE SAME SITUATION YOU DESCRIBED ABOUT BOTTLENECKS. FOR EXAMPLE, IF YOU ASSEMBLED A TEAM AROUND -- I'M JUST GOING TO PICK IT OUT OF THE AIR WITH NO SPECIFICITY -- CARDIOVASCULAR DISEASE, AND IF YOU IDENTIFY THE BOTTLENECKS, AND IF THAT TEAM SPECIFICALLY WORKED ON BOTTLENECKS, THAT COULD

POTENTIALLY HAVE A LOT OF ADVANTAGE TO MANY OTHER RESEARCHERS AND TEAMS ASSEMBLING TO WORK ON A THERAPY SPECIFIC.

SO THAT WAS DEFINITELY RECOGNIZED. ARLENE, I DON'T REMEMBER WHERE WE CAME OUT ON WHERE THAT FITS INTO OUR PROGRAMS. WE FELT IT FIT.

DR. CHIU: THIS IS NOT THE ONLY PROGRAM THAT TARGETS DISEASES, BUT WE CERTAINLY HEARD THAT THERE WERE MANY OTHER APPROACHES, SOME OF WHICH MAY NOT LEAD DIRECTLY TO AN IND AS WE PICTURED HERE, BUT WILL ADDRESS BOTTLENECKS. AND WE SAW IN THE STRATEGIC PLAN THAT THERE IS ALSO A DIFFERENT PROGRAM CALLED TRANSLATIONAL RESEARCH WHERE THEY COULD NOW TARGET A TEAM, FOR RESEARCH TEAMS, TARGET CERTAIN ASPECTS. SOMEBODY BROUGHT UP THE IDEA OF DELIVERY OF CELLS. IT DOESN'T HAVE TO BE FOR ANY PARTICULAR DISEASE, BUT JUST THE CONCEPT OF DELIVERY, SAFE DELIVERY, UTENSILS FOR DELIVERY, APPROACHES AND MEASURES IS ANOTHER RESEARCH IDEA.

THOSE WOULD COME, WE THINK, AFTER THE DISCUSSION THAT MIGHT FIT BETTER UNDER THE TRANSLATIONAL RESEARCH PROGRAM SO THAT WE WILL HAVE DIFFERENT SORT OF NARROWER FOCUS FOR EACH PROGRAM AND TARGET THOSE IN SLIGHTLY DIFFERENT WAYS. WE DO WANT TO COVER THE WATERFRONT. AND SO IF PEOPLE PROPOSE SUCH THINGS, WE WANT TO ACCOMMODATE IT THROUGH PROGRAMS THAT ARE DESIGNED

AROUND THAT KIND OF APPROACH, FOR EXAMPLE.

DR. STEFFEN: IF THERE ARE NO MORE COMMENTS, WE'LL MOVE ON. I PROMISE YOU YOU WILL HAVE THE OPPORTUNITY AGAIN.

WE'RE MOVING TO TEAM COMPOSITION. AND WE HEARD THREE KEY MESSAGES HERE. THE DIVERSE SET OF SKILLS IS NEEDED TO DEVELOP THE STEM CELL-BASED THERAPIES, AND I THINK WE'RE HEARING EVIDENCE OF THAT IN OUR OWN DISCUSSION HERE TODAY. AND PARTICIPANTS ALSO WANTED US TO RECOGNIZE THAT PERSONNEL MAY NOT ALL BE AVAILABLE WITHIN A SINGLE ORGANIZATION AT ANY ONE TIME.

THE SECOND POINT THEY BROUGHT UP WAS THAT AN ENGAGED AND ENERGETIC LEADER IS ESSENTIAL FOR TEAM RECRUITMENT, MOTIVATION, AND SUCCESS. WE'LL TALK ABOUT SOME QUALITIES THEY IDENTIFIED FOR THE LEADER.

AND THIRD, A PROJECT MANAGER WAS IDENTIFIED AS HIGHLY DESIRABLE TO HELP COORDINATE THESE LARGE TEAMS OF MULTIDISCIPLINARY INVESTIGATORS. WE'LL GO THROUGH THESE ON THE NEXT FEW SLIDES.

YOU SHOULD BE LOOKING AT A LIST OF TEAM EXPERTISE. AND THESE WERE THE TYPES OF EXPERTS IDENTIFIED THAT WOULD BE INVOLVED IN TRANSLATIONAL PROJECTS. AND STARTING IN THE UPPER LEFT CORNER ARE THE MORE BASIC AND DISCOVERY-ORIENTED RESEARCHERS, MOVING DOWN AND TO THE RIGHT IS THE EXPERTISE THAT IS NEEDED FOR

CLINICAL TRIALS. AND ULTIMATELY THE TEAM COMPOSITION WOULD DEPEND ON THE SCOPE OF THE PROJECT, THE OBJECTIVES, AND THE DISEASE ITSELF, AND NOT ALL PROJECTS WOULD NECESSARILY NEED ALL THESE PARTICIPANTS.

THE OTHER OBSERVATION THAT THE PARTICIPANTS MADE IS THAT TEAMS WOULD BE DYNAMIC AS THE PROJECT PROGRESSED CLOSER TO CLINICAL TESTING. AS AN EXAMPLE, YOUR REGULATORY SPECIALIST MIGHT BE AN OBSERVER IN THE VERY EARLY STAGES AND PROVIDE SMALL, BUT IMPORTANT INPUT; BUT TOWARD THE END, AS YOU MOVE TOWARD CLINICAL TRIALS, THEIR INPUT WOULD BE CRITICAL TO MOVING THE PROJECT ALONG.

ON LEADERSHIP WE HEARD FROM OUR NIH REPRESENTATIVE THAT THE SUCCESS OF THE TRANSLATIONAL PROGRAM THERE DEPENDS ON THREE THINGS: ONE, THE TEAM'S EXPERIENCE WITH THERAPEUTIC DISCOVERY AND DEVELOPMENT; TWO, THE INVOLVEMENT OF THE AGENCY AND STAFF; AND, THREE, THE COMMITMENT OF THE PI. SO WE IDENTIFY AS AN IMPORTANT LEADERSHIP CHARACTERISTIC COMMITMENT OF THE TEAM LEADER. AND IN ORDER TO ATTRACT THE TOP LEADERS, STABLE FUNDING IS CRITICAL.

WE HEARD FROM OUR PARTICIPANTS THAT THESE LONGER TERM PROJECTS MIGHT BE MORE RISKY AND NEED TO SHARE A LOT OF THE SUCCESSES AMONG PEOPLE SO THAT THE ONE THING THAT COULD ATTRACT THE TOP LEADERS WAS ACTUALLY THE

LENGTHY, STABLE FUNDING.

AND, FINALLY, IN ORDER TO COMMAND RESPECT FROM THE TEAM, THE LEADER WOULD NEED TO BE A PRACTICING SCIENTIST WHO'S IN GOOD STATURE AND WHOSE LAB IS INVOLVED IN A PROJECT AND WHO ORCHESTRATES RATHER THAN DICTATES THE DEVELOPMENT OF THE RESEARCH PLAN AND WHO IS A LEADER AMONG EQUALS. AND THIS IS REALLY DESCRIBED AS SOMEONE WHO IS MOTIVATED BY THE MISSION OF THE PROJECT RATHER THAN THEIR OWN INTEREST.

WE HAD MUCH DISCUSSION ON THE CONCEPT OF ACTIVE MANAGEMENT AND OVERSIGHT OF THESE PROJECTS. AND WHAT WE CONSISTENTLY HEARD IS THAT, ONE, THERE'S A ROLE FOR A PROJECT MANAGER AND THAT EXECUTIVE COMMITTEES SHOULD BE INVOLVED. AND, HOWEVER, I WILL TELL YOU FOR EVERY TIME WE HEARD THE PROJECT MANAGER AND THE EXECUTIVE COMMITTEES INVOLVED, THE ROLES, RESPONSIBILITIES, AND COMPOSITION VARIED SIGNIFICANTLY AMONG THE TEAMS AND ORGANIZATIONS WHO PRESENTED THEIR MODELS.

EXECUTIVE COMMITTEES, THERE MIGHT BE TWO TYPES. AN ADVISORY COMMITTEE MIGHT BE APPOINTED BY THE TEAM, BE VERY SCIENTIFIC IN NATURE, AND CAN HELP THE RESEARCH PROGRESS IN A DESIRED DIRECTION. IN THE OVERSIGHT COMMITTEES, MOST OF THE MODELS THAT WE HEARD WERE THIRD-PARTY MEMBERS AND MIGHT BE EXPERTS IN THE AREA AND MIGHT BE ASSEMBLED BY THE FUNDING AGENCY TO HELP WITH

OVERSIGHT AND EVALUATION.

IN ORDER FOR THE MANAGEMENT AND OVERSIGHT MECHANISMS TO BE EFFECTIVE, WE HEARD THAT THERE NEEDS TO BE CLOSE COMMUNICATION BETWEEN PROJECT LEADERSHIP AND THE FUNDING AGENCY. WE ALSO HEARD LOUD AND CLEAR THAT IT SHOULD NOT BE BURDENSOME WRITTEN COMMUNICATION, BUT POSSIBLY SOMETHING LIKE WEEKLY TELECONFERENCES.

FINALLY, PERIODIC EVALUATIONS AGAINST A PROJECT PLAN AND MILESTONES WOULD NEED TO BE CONDUCTED. AND THESE EVALUATIONS WOULD HAVE THREE POSSIBLE OUTCOMES. THERE WOULD BE THE POTENTIAL TO MOVE SUCCESSFUL PROJECTS FURTHER ALONG DOWN TOWARD THE CLINIC. THERE WOULD BE REORIENTING OF PROJECTS, IF FEASIBLE. AND FINALLY, FAILURE TO MEET CRITICAL MILESTONES COULD RESULT IN PROJECT TERMINATION.

NOW, WHAT WE GOT FROM THE SENSE OF THE PARTICIPANTS IN THE ROOM, THAT THIS IS A DEPARTURE FROM TRADITIONAL GRANT FUNDING, BUT IT'S NOT UNCOMMON IN INDUSTRY.

THE OTHER TAKE-HOME MESSAGE IS THAT THIS CONCEPT OF ACTIVE MANAGEMENT AND DOING IT WELL IS VERY RESOURCE INTENSIVE.

I'VE MOVED ON TO RESOURCES. AND BEYOND PEOPLE AND RESEARCH BUDGETS, WE ASKED THE TEAMS WHAT THEY WOULD NEED. WE HEARD SOME TEAM-SPECIFIC RESOURCES, WHICH MIGHT

INCLUDE COMMUNICATION TECHNOLOGIES AND DATA SHARING TECHNOLOGIES. WE WERE GIVEN EXAMPLES OF TEAM RESEARCH WEBSITES, NETWORKS THAT USED VIDEOCONFERENCING, NOT ONLY FOR THEIR SCIENTIFIC MEETINGS, BUT ALSO FOR THEIR EVALUATION MEETINGS, AND THE NEED FOR DISTRIBUTED DATA. WE HEARD STEM CELL SPECIFIC RESOURCES, CORE SERVICES, AND I THINK WE HEARD TWO MORE EXAMPLES TODAY AROUND ASSISTANCE WITH THE IRB AND THE IACUC, GMP, GLP, AND REGULATORY SERVICES ALSO CAME UP FREQUENTLY FROM THE PARTICIPANTS.

AND FINALLY, IT WAS IDENTIFIED THAT THE REGULATORY REQUIREMENTS REALLY DRIVE MANY OF THE PRECLINICAL PROJECT DEVELOPMENT NEEDS. AND I THINK THE ISSUE HERE WAS NOT JUST RECOGNIZING THAT REGULATORY DRIVES THE REQUIREMENTS, BUT ALSO THAT THE REGULATORY EXPERTISE, IN PARTICULAR IN TAKING CELLULAR THERAPY PRODUCTS TO TRIAL, IS VERY, VERY LIMITED AT THIS STAGE IN THIS EARLY DEVELOPING FIELD.

FINALLY, WE DISCUSSED OPERATIONAL CHALLENGES FOR THESE MULTIDISCIPLINARY AND POSSIBLY INTERINSTITUTIONAL TEAMS. AND THE FIRST POINT THAT THE PARTICIPANTS BROUGHT UP WAS THAT THE METRICS FOR SUCCESS AND REWARD SYSTEMS ARE DIFFERENT FOR TEAM PROJECTS COMPARED TO INDIVIDUAL SCIENTIST PROJECTS. AN EXAMPLE THEY GAVE WAS THAT INDIVIDUAL SCIENTISTS GET MEASURED BY

THE NUMBER OF R01 GRANTS AND THE PRIME PUBLICATIONS THAT THEY BRING IN TO THE INSTITUTION VERSUS TEAMS WHICH NEED TO BE MORE INCENTED TO ACHIEVE A TEAM GOAL.

SO, THEREFORE, THEY ADVISED US THAT GUIDELINES GOVERNING DATA SHARING AND PUBLICATION SHOULD BE DEFINED UP FRONT. SOME CONSORTIA PUBLISH ALPHABETICALLY, SOME PUBLISH UNDER A GROUP NAME, AND OTHERS CARVE UP THE PROJECT INTO AREAS OF INDIVIDUAL LEADERSHIP FOR ELEMENTS. SO THE MESSAGE WE TOOK FROM THAT IS NOT THAT WE SHOULD LISTEN TO WE CAN'T DO THAT, BUT OFFER SOME OF THESE POSSIBILITIES AS WAYS TO SUPPORT TEAM-BASED RESEARCH.

AND FINALLY, TEAMS NEED TO ESTABLISH WELL-DEFINED RULES REGARDING INTELLECTUAL PROPERTY OWNERSHIP UP FRONT. AND THIS WILL BE ESPECIALLY TRUE IF WE ENCOUNTER NOT-FOR-PROFIT AND FOR-PROFIT PARTNERSHIPS, WHICH DOES HAVE A PRECEDENT IN SMALL MOLECULE DRUG DEVELOPMENT, SO WE CAN ANTICIPATE THAT WE MIGHT SEE TEAMS FORMING LIKE THAT.

THAT'S THE CONCLUSION OF OUR PRESENTATION ON THE WORKSHOP FINDINGS, AND WE'D LIKE TO INVITE DISCUSSION AND COMMENTS ON THE REPORT.

DR. CHIU: FOR THE GRANTS WORKING GROUP, ARE THERE ANY THOUGHTS OR COMMENTS ON THE SUMMARY OF THE WORKSHOP OR ANY GUIDANCE THAT WE CAN GET FROM YOU?

CHAIRMAN ORKIN: I THINK IT'S QUITE A

THOUGHTFUL SUMMARY. I THINK BEST COMMENTS WILL COME FROM THOSE WHO HAVE ACTUALLY DEALT WITH IND'S AND IRB'S INTIMATELY AND CAN PROVIDE SOME DIRECT INPUT.

DR. CHIU: I SHOULD MENTION THAT ALL OF YOUR THOUGHTS AND COMMENTS WILL BE INCLUDED -- WE HAVE A PRELIMINARY REPORT WITH MORE DETAIL OF THE WORKSHOP THAT IS IN THE WORKS. WE WOULD LIKE TO INCLUDE ANY COMMENTS FROM YOU OR FROM THE PUBLIC NOW REGARDING DISEASE TEAMS THAT WE CAN INCLUDE AS A POSTSCRIPT TO THAT REPORT. AND THAT REPORT WILL BE PRESENTED TO THE ICOC AS WELL AS MADE PUBLIC ON OUR WEBSITE WHEN IT IS READY.

DR. CSETE: THIS IS MORE AN ASIDE. I JUST WANT TO SAY THAT THIS RFA, WHEN IT COMES OUT, IS EXACTLY THE REASON WHY I AGREED TO BE ON THE GRANTS WORKING GROUP.

DR. CHIU: ALL RIGHT.

DR. CSETE: AS A SCIENTIST, THIS IS THE MOST EXCITING THING TO SEE. I THINK YOU GUYS ARE IN A POSITION TO REALLY PUSH THIS IN A WAY THAT OTHER BODIES HAVE NEVER BEEN ABLE TO DO.

DR. CHIU: I'M DELIGHTED TO HEAR THAT. ALSO, BECAUSE IT IS SO NEW AND WE'RE SORT OF TESTING THE WATERS, YOU SAW WHAT BETTINA SAID, THAT WE'RE GOING TO FIRST COME OUT AND ASK THE ICOC FOR APPROVAL OF PLANNING GRANTS, WHICH ARE SMALL, ONE-TIME-ONLY FUNDING SO THAT PEOPLE CAN ASSEMBLE TEAMS AND MOVE THEM FORWARD AND

GATHER ALL THIS IN PREPARATION FOR A REALLY RESOUNDINGLY SOLID, WELL-THOUGHT OUT PROPOSAL.

AND THE OTHER THING THAT WE'RE TRYING TO THINK ABOUT IS IN THE BEGINNING TO MOVE CAUTIOUSLY AND ONLY AWARD FOR THE BIG, LONG-TERM DISEASE TEAM GRANTS WHICH WILL COME TO YOU FOR REVIEW, ONLY THE VERY BEST.

SO THESE PLANNING GRANTS, WE HOPE, WOULD FERTILIZE A LOT OF IDEAS. AND EVEN IF THEY DON'T EVENTUALLY END UP WITH A DISEASE TEAM GRANT FROM US, THEY MAY LAUNCH DISEASE TEAM GRANTS TO OTHER FUNDING ENTITIES, THE NIH OR OTHER FOUNDATIONS, FOR EXAMPLE. SO WE HOPE WE CAN GENERATE A LOT OF THOUGHT ABOUT DISEASE TEAMS.

DR. WILLIAMS: I THINK THIS IS ACTUALLY QUITE GOOD AND VERY COMPREHENSIVE. I GUESS I WOULD ADD ONE THING THAT'S NOT TOUCHED ON QUITE AS MUCH HERE MAYBE. ONE OF THE PROGRAMS THAT WE INITIATED HERE AT CINCINNATI WAS ACTUALLY A SPECIAL TRIALS OFFICE THAT DEVELOPED EXPERTISE IN IND'S AND DISCUSSIONS WITH THE FDA AND ESAP AND RAC FOR GENE THERAPY. AND THAT HAS BECOME ENORMOUSLY HELPFUL TO SCIENTISTS. AND, IN FACT, I THINK WE'RE NOW SUPPORTING 17 IND'S OVER THE LAST FOUR YEARS.

IT JUST PROVIDES A LEVEL OF COMFORT FOR MORE BASIC SCIENTISTS WHO HAVEN'T EVER WORKED IN HUMAN TRIALS TO HAVE A LOCAL OFFICE THAT'S QUITE COMFORTABLE TALKING WITH THE FDA ON LOTS OF DIFFERENT ISSUES. AND IT MIGHT

BE A PARADIGM THAT COULD BE THOUGHT ABOUT IN THE CONTEXT OF HOW YOU'RE ALSO SETTING UP THIS PROGRAM.

AND I THINK, IN ADDITION TO WHAT YOU'VE ALREADY GOT IN YOUR SLIDES ON TEAM BUILDING, I THINK IN HAVING DONE NOW QUITE A FEW GENE THERAPY TRIALS, IT STRIKES ME THAT ONE OF THE THINGS THAT'S HELPED THE MOST IN OUR TRIALS IS TO HAVE A DISEASE-SPECIFIC SCIENTIST WITH A LOT OF EXPERTISE IN A PARTICULAR DISEASE PAIRED WITH SOMEONE WHO UNDERSTANDS THE TECHNOLOGY AT THE BASIC LEVEL. AND THAT BEING THE LEADERSHIP PART OF THE TEAM BASICALLY.

DR. CHIU: A TWO-LEADER, ONE COVERING DIFFERENT AREAS, THEN, IN THE TEAM.

DR. WILLIAMS: I THINK THE TECHNOLOGIES AND FURTHER TYPES OF THINGS THAT YOU'RE TRYING TO SUPPORT HERE ARE IN AND OF THEMSELVES QUITE COMPLICATED. AND ON THE OTHER HAND, FOR MANY DISEASES, THE DISEASES THEMSELVES ARE QUITE COMPLICATED. AND SO HAVING -- IF YOU THINK ABOUT IT, HAVING ONE PERSON WHO'S GOING TO HAVE THE BREADTH AND DEPTH OF KNOWLEDGE IN BOTH THOSE AREAS IS UNLIKELY.

DR. CSETE: I THINK IF YOUR LIST OF MEMBERS OF TEAMS IS MEANT TO BE INCLUSIVE, THEN ONE THING TO CONSIDER ADDING THERE IS -- THERE'S ALL THIS WONDERFUL MODELING AND SIMULATION COMING OUT OF SYSTEMS BIOLOGY. SO STATISTICIAN DOESN'T REALLY COVER IT. SO JUST AS

ENGINEERS OR OTHER MATHEMATICIANS OR SOMETHING SHOULD BE PART OF THIS LIST AS WELL.

DR. CHIU: THANK YOU. DR. WILLIAMS, COULD YOU PROVIDE US WITH A LITTLE BIT MORE INFORMATION ABOUT THE SPECIAL TRIALS OFFICE? HOW MANY PEOPLE ARE IN THERE? AND WHAT KINDS OF EXPERTISE DO THEY HAVE JUST TO GIVE US A BETTER IDEA?

DR. WILLIAMS: IT'S VERY SIMILAR TO WHAT MANY INSTITUTIONS HAVE, I THINK, CLINICAL TRIALS OFFICE, ESPECIALLY FOR CANCER CENTERS. HERE AT CINCINNATI IT HAS NOW, I THINK, UP TO EIGHT FTE'S, AND THAT INCLUDES REGULATORY EXPERTISE, MEDICAL WRITER, AND RESEARCH NURSE AND RESEARCH COORDINATORS, AND THEN BIOSTATISTICS. AND THAT'S VERY SIMILAR TO MANY OTHER CLINICAL TRIALS OFFICES EXCEPT SMALLER.

AND THE WAY WE HAVE FOCUSED IT HERE IS, BECAUSE WE DO HAVE A CLINICAL TRIALS OFFICE, IS THAT WE SORT OF SEPARATE OUT THE NEED FOR AN IND AS BEING WHAT YOU NEED TO GET INTO THIS OFFICE FOR ASSISTANCE. IF YOU ARE GOING TO NEED AN IND, THEN THIS IS WHERE YOU GO. SO THE REGULATORY EXPERTISE IN THIS PARTICULAR OFFICE IS HEAVILY ON THE SIDE OF DEALING WITH THE FDA AND DEALING WITH CTAP AND RAC AND THAT TYPE OF THING, THE NIH RECOMBINANT DNA ADVISORY COMMITTEE.

DR. CHIU: I WAS JUST WRITING IT DOWN.

DR. BONNER-WEIR: I KNOW OF THINGS LIKE THE IMMUNE TOLERANCE NETWORK, WHICH IS FUNDED BY NIH. AND I THINK YOU CAN CALL IT SOME OTHER FOR PRIVATE INSTITUTIONS. BUT IT OVERSEES A NUMBER OF CLINICAL TRIALS FOR DIABETES. AND ONE OF THE THINGS THAT THEY HAVE, THEY HAVE AN OFFICE IN THAT NETWORK. SO IT MAY BE THAT IF YOU HAD THIS SORT OF OFFICE WITHIN CIRM FOR THIS EXPERTISE SO THAT DIFFERENT PLACES, BECAUSE THE STEM CELLS THEMSELVES ARE GOING TO HAVE A NUMBER OF DIFFERENT REGULATORY ISSUES, MANY OF WHICH HAVE NOT BEEN WORKED OUT AS YET, BUT IF YOU HAD FOCUSED ON THAT OR ONE PLACE THAT WAS SORT OF THE RESOURCE OF THE STATE OF CALIFORNIA, THAT MAY HELP THINGS EVEN FURTHER.

DR. CHIU: SO A MORE CENTRALIZED RESOURCE FOR ALL CALIFORNIA INVESTIGATORS, KIND OF.

DR. BONNER-WEIR: RIGHT. OBVIOUSLY YOU STILL COULD HAVE THESE LOCAL, BUT THE WHOLE ISSUE ABOUT CELL -- THE REGULATIONS ABOUT CELL-BASED THERAPY IS REALLY NEW AND HASN'T REALLY BEEN WORKED OUT. I KNOW IN THE ISLET TRANSPLANTATION FIELD, THEY HAVE BEEN WORKING SORT OF TRYING TO WHAT DO YOU NEED. FDA IS TRYING TO FIGURE OUT WHAT THEY NEED.

CHAIRMAN ORKIN: ANY OTHER COMMENTS?

DR. STEWARD: THIS IS SORT OF A QUESTION FOR CIRM. THIS LATEST DISCUSSION, I THINK, I FIND VERY

INTERESTING, THE IDEA OF SOME SORT OF CENTRALIZED GROUP THAT IS RESPONSIBLE FOR REGULATORY KINDS OF ISSUES. JUST FOR THOSE OF YOU WHO AREN'T FAMILIAR, ONE OF THE BIG PROBLEMS WITH CIRM IS THAT THERE'S A VERY TIGHT LIMIT TO THE TOTAL NUMBER OF PEOPLE THAT CAN ACTUALLY BE IN CIRM HEADQUARTERS. THE NUMBER IS 50. AND SO IT COULD NOT BE PART, PRESUMABLY, OF THE CIRM ORGANIZATION ITSELF.

MY QUESTION IS IS THIS A TEAM THAT COULD BE CONSIDERED FOR FUNDING, A REGULATORY AFFAIRS TEAM, LET'S CALL IT?

DR. CHIU: THAT'S A VERY INTERESTING CONCEPT THAT WE CAN --

MR. KLEIN: I THINK WHAT YOU'RE SUGGESTING, OS, IS THE MOST PROBABLE DIRECTION WE WOULD NEED TO GO IS CONTRACTING FOR A TEAM, AND THEY COULD RESPOND. WE COULD HAVE A COMPETITION, IN FACT, FOR A TEAM OR MULTIPLE TEAMS, GIVEN WE HAVE A BIG STATE, BECAUSE ALL TEAMS MIGHT NOT BE -- TO HAVE ONE CENTRALIZED TEAM, WE MAY NOT HAVE THE DIVERSITY OF EXPERIENCE ON IT THAT WE MIGHT NEED. WE MIGHT NEED A COUPLE OF TEAMS IN THE STATE THAT SERVE AS RESOURCE CENTERS, BUT ACT ON CONSULTING CONTRACTS BECAUSE, AS YOU QUITE APPROPRIATELY POINT OUT, WE CAN'T INTERNALIZE THEM AS STAFF AT CIRM.

DR. WILLIAMS: SOMEONE MADE A COMMENT ABOUT ONE OF THE DIABETES TEAMS HAVING THIS SETUP. SO, AGAIN, THIS

CORE THAT I SPOKE ABOUT IN CINCINNATI ACTUALLY COMPETED FOR AND GOT AWARDED FOR THE NIH RARE LUNG DISEASES CONSORTIUM THE CENTRAL REGULATORY OFFICE FOR MULTIPLE INSTITUTIONS AROUND THE COUNTRY DOING THESE KINDS OF STUDIES. SO YOU COULD HAVE, AS PART OF YOUR RFA, IF YOU WANTED AND YOU DIDN'T WANT TO EMBED THESE EXTRA FTE'S IN YOUR OWN OFFICE, YOU COULD HAVE THAT AS PART OF THE TEAM APPROACH, THAT PEOPLE COULD COMPETE FOR SETTING UP A REGULATORY OFFICE THAT WOULD ASSIST EVERYBODY IN ISSUES DEALING WITH REGULATORY OVERSIGHT AND INTERACTIONS ALONG THESE LINES WITH EITHER NIH OR MORE APPROPRIATELY WITH THE FDA.

DR. CHIU: THAT'S RIGHT. JUST LIKE WE HAVE COMPETED FOR SHARED LABS OR TECHNIQUES COURSES, WE COULD ALSO COMPETE OUT A RESOURCE SUCH AS THIS FOR THE STATE. GREAT. I WILL TRY AND FIND OUT MORE ABOUT HOW THE NIH STRUCTURED THIS IN THEIR RARE LUNG DISEASE CONSORTIUM, BUT THIS IS A VERY GOOD LEAD. THANK YOU.

MR. KLEIN: EXCELLENT IDEA.

DR. CHIU: ARE THERE ANY COMMENTS FROM THE PUBLIC? WE WOULD LOVE TO HEAR FROM THE PUBLIC ANY THOUGHTS THEY MIGHT HAVE.

DR. STEFFEN: IF NONE, NO MORE COMMENTS --

MR. KLEIN: WHILE YOU'RE WAITING FOR THE PUBLIC, BETTINA AND ARLENE AND THE REST OF YOUR

SCIENTIFIC GROUP THERE, THIS IS BOB KLEIN, I'D JUST LIKE TO SAY -- TO ECHO SOME OF THE SCIENTISTS' COMMENTS, THAT I THINK THIS WAS AN EXCELLENT PRESENTATION AND WILL REALLY BE AN INVALUABLE RESOURCE FOR THOSE WHO ARE GOING TO COMPETE FOR THE PLANNING GRANTS TO REALLY SCOPE AND ORGANIZE THEIR TASK.

DR. CHIU: WE HOPE SO.

DR. STEFFEN: THANK YOU. IF THERE ARE NO COMMENTS ON THE REPORT, WE WOULD LIKE TO MOVE TO DISCUSSION QUESTIONS FOR PROPOSALS. AND THE FIRST QUESTION THAT WE HAVE FOR THE GRANTS WORKING GROUP IS HOW WOULD ONE ASSESS A DISEASE TEAM PROPOSAL?

DR. CHIU: THIS IS A LEADING QUESTION, AND WE HOPE THAT WE WILL HAVE A FEW THOUGHTS FROM YOU BEFORE WE WRITE THE INITIATIVE. THIS WOULD GREATLY HELP US. IF, YOU KNOW, TEN TEAMS CAME IN, EACH ON A DIFFERENT DISEASE WITH A DIFFERENT APPROACH, WHAT DO YOU THINK WE SHOULD, YOU KNOW, SAY IS CRITICAL?

DR. CSETE: WELL, THIS IS ONE AREA WHERE FEASIBILITY VERSUS NOVELTY MAYBE SHOULD GO ON THE FEASIBILITY SIDE. AGAIN, JUST THINKING OF SOMEONE WHO TRAVERSES HOSPITALS EVERY DAY, SO WHAT YOU'RE REALLY ASKING US IS WHAT MAKES THIS KIND OF APPLICATION DIFFERENT THAN HOW WE WOULD REVIEW AN NIH GRANT OR A REGULAR R01 APPLICATION. AND I THINK FEASIBILITY HAS TO

BE VERY HIGH UP.

DR. BRIVANLOU: I ALSO WANT TO SECOND THAT, BUT ALONG THE RANGE OF PROJECTS THAT ARE FEASIBLE, I WILL VOTE FOR THE ONES THAT I WOULD CALL SCIENTIFICALLY READY OR MORE MATURE THAN OTHERS. I THINK THE FIELD DESPERATELY NEEDS ONE DISEASE TO BE ADDRESSED IN A POSITIVE FASHION WITH CELL-BASED THERAPY OF STEM CELLS OR ANY APPROACHES THAT USES STEM CELLS TO GET TO THAT THERAPY.

AND I THINK AFTER THE FIRST ONE, EVERYTHING IS GOING TO BECOME MORE EASY, NOT ONLY IN TERMS OF THE SOCIOPOLITICAL INTERFACE OF IT, BUT ALSO IN TERMS OF OUR UNDERSTANDING OF THE SCIENTIFIC ENDEAVOR. SO AMONG ALL THE PROJECTS THAT ARE FEASIBLE, THERE ARE SOME THAT ARE VERY, VERY NEAR TO COMPLETION. THEY MIGHT NOT BE THE SEXIEST ONE OR THE ONES THAT ARE THE MOST POPULAR, LIKE PARKINSON'S OR OTHERS, BUT REALLY ARE THE ONES THAT ARE VERY, VERY CLOSE TO GET A RESULT. I WOULD PERSONALLY AMONG THE FEASIBLE ONE WOULD VIEW IT AS THE ONE THAT I FEEL THE PRELIMINARY RESULT IS SO STRONG, THAT THE STEPS INVOLVED TO THE FINAL INFLUENCE ARE MINIMAL.

DR. CHIU: RIGHT.

MR. KLEIN: HOW WOULD YOU EVALUATE THE IMPORTANCE OF THE QUALITY AND DEPTH OF THESE BOTTLENECKS AND CHALLENGES AND POTENTIAL SOLUTIONS TO THOSE

BOTTLENECKS AND CHALLENGES? BECAUSE IT WOULD SEEM THAT IF THE PROPOSAL HAD AN EXTREMELY GOOD HANDLE ON WHERE THE MAJOR PROBLEMS MIGHT DEVELOP, THAT MIGHT BE AN IMPORTANT PART OF THE EVALUATION AS REFERENCED MUCH EARLIER, I THINK, BY DR. MURPHY.

CHAIRMAN ORKIN: I THINK THAT'S TRUE. THAT WOULD, I THINK, FALL UNDER FEASIBILITY TO A CERTAIN EXTENT. IN ADDITION, I THINK ONE WOULD ALSO WANT TO HAVE SOME WAY TO -- THIS MAY BE DIFFICULT, BUT SOME WAY TO ASSESS THE INTEGRATION OF THE TEAM ITSELF TO BE CERTAIN THAT THIS IS A TEAM THAT IS COMFORTABLE WORKING TOGETHER, HAS ONE GOAL IN MIND RATHER THAN A BUNCH OF FOLKS WHO HAVE BEEN ORGANIZED SOLELY FOR THE APPLICATION, SAKE OF THE APPLICATION.

DR. CSETE: IN TERMS OF THE ASSESSMENT, I THINK THAT WE MAY BE FACED AS A WORKING GROUP WITH DISEASES FOR WHICH THERE'S NOT INTERNAL CLINICAL EXPERTISE. AND I THINK THE WORKING GROUP FOR THESE SHOULD HAVE THE ABILITY TO CALL IN PERHAPS CONSULTANTS WHO'S THE EXPERT, IF NEEDED.

DR. CHIU: SUBJECT EXPERTS. ABSOLUTELY WE NEED THAT.

JUST TO GO BACK TO SOME OF THE POINTS, PARTICULARLY DR. BRIVANLOU'S POINT AND ALSO MR. KLEIN'S POINT, I GUESS ONE OF THE THINGS MIGHT BE, AND I'M ASKING

FOR YOUR OPINION, THAT IF IT'S MILESTONE DRIVEN, THAT ANTICIPATION OF POTENTIAL PROBLEMS AND PITFALLS AND SOLUTIONS TO HOW THOSE COULD BE OVERCOME AND WHEN IT WOULD BE A DROP-DEAD SORT OF END MIGHT BE ANOTHER WAY OF INCREASING THE RIGOR OF SOME OF THESE PLANS. WHAT DO YOU THINK?

CHAIRMAN ORKIN: SOUNDS LIKE AN EXCELLENT IDEA.

DR. BRIVANLOU: I AGREE.

DR. CSETE: YEAH.

DR. STEFFEN: I THINK WE ADDRESSED COMPARING AND EXPERTISE. ARE THERE ANY MORE COMMENTS ON HOW THESE PROPOSALS MIGHT BE COMPARED? I THINK WE'VE ADDRESSED EXPERTISE.

DR. CHIU: SOME OF YOU MEMBERS OF THE WORKING GROUP MAY HAVE COME ACROSS SOMETHING SIMILAR TO WHAT WE'RE PROPOSING, AND I WONDERED IF YOU HAVE SOME THOUGHTS TO SHARE WITH US AS TO STRENGTHS AND WEAKNESSES OF SOME OF THE THINGS THAT HAVE COME ACROSS YOUR DESK THAT WE SHOULD WATCH OUT FOR.

DR. BRIVANLOU: IN MY EXPERIENCE IN NEW YORK, THE MOST CHALLENGING FOR US HAS ACTUALLY BEEN THE INTEGRATION OF THE DIFFERENT VARIABLES THAT ARE REQUIRED TO DO THIS SUCCESSFULLY. THIS IS DOWNSTREAM OF GETTING ALL THE VALUABLE PEOPLE AND CONSULTANT AND EVERYTHING. THERE IS A VERY STRONG, AS YOU ALREADY HEARD BEFORE,

ADMINISTRATIVE ASPECT AND BUREAUCRATIC ASPECT TO THIS THAT IS QUITE COMPLEX, NOT NECESSARILY BECAUSE THE RULES ARE NOT VERY WELL DEFINED, ALSO IT'S BECAUSE IT'S DIFFICULT TO INTEGRATE THIS IN A HOMOGENEOUS WAY.

WE TALKED ABOUT LEARNING FROM EXPERIENCES OF OTHER UNIVERSITIES. I GUESS THERE ARE MANY DIFFERENT FLAVORS OF THE RULES RIGHT NOW AMONG UNIVERSITIES. IT'S VERY DIFFICULT TO COME UP WITH A CONSENSUS. IT'S VERY DIFFICULT TO ANSWER THIS QUESTION. I THINK THE BEST WAY TO DO IT IS TO PICK COUPLE OF THEM THAT THE TEAMS ARE SCIENTIFICALLY MATURE AND PROVIDE YOU WITH THE BEST CHANCE OF SUCCESS, AND THEN GO TO THEM IN A MORE OR LESS PREPARED, BUT IMPROVISED WAY AND SOLVE IT AS YOU GO FORWARD.

I THINK THAT THERE ARE SO MANY ELEMENTS INVOLVED, THAT IT'S VERY DIFFICULT TO PREDICT AT THIS POINT WHERE THE REAL BOTTLENECKS ARE GOING TO BE. I PERSONALLY FROM MY OWN EXPERIENCE, THE BOTTLENECKS ARE NOT SCIENTIFIC USUALLY.

DR. CSETE: I'M WONDERING IF YOU DISCUSSED THE POTENTIAL NEED FOR SITE VISITS. AS EXPERIENCE, SOMETIMES WHEN I'VE BEEN ON A SITE VISIT FOR A PROJECT OR SOMETHING LARGER, YOU REALLY GET A SENSE OF WHETHER PEOPLE HAVE TALKED TO EACH OTHER OR NOT. AND PART OF THIS WHOLE -- A BIG PART OF WHAT YOU TALKED ABOUT WAS HOW A LEADER WAS

GOING TO BE THE GLUE FOR THIS TEAM, AND SOMETIMES ON-SITE CAN GET A MUCH BETTER SENSE OF THAT.

DR. CHIU: RIGHT. ONE THING WE HAD HEARD, ESPECIALLY FROM THE NIH REP, WAS THE CLOSE COORDINATION, FOR A SCIENTIFIC MEMBER OF THE INSTITUTE TO BE VERY CLOSE IN PROJECT MANAGEMENT SO THEY KNOW WHAT'S HAPPENING IN TERMS OF COMMUNICATION. BUT A SITE VISIT SURELY WILL BRING A CLEARER IDEA OF HOW THINGS ARE DONE AND WHERE THEY'RE DONE. YOU'RE RIGHT.

I HAD ANOTHER QUESTION, IF THERE ARE NO MORE POINTS ABOUT THIS, IS HOW LONG DO YOU THINK THESE TYPES OF GRANTS SHOULD BE FUNDED FOR?

DR. WILLIAMS: IT DEPENDS ON HOW YOU, AGAIN, SET IT UP. BUT MY -- IN FACT, I JUST WROTE AN EDITORIAL FOR *MOLECULAR THERAPY* ABOUT THE NIH FUNDING OF CLINICAL TRIALS. THE POINT I TRIED TO MAKE IN THAT EDITORIAL WAS THAT TO BE REALLY GOOD, THE FUNDING HAS TO BE OVER A NUMBER OF YEARS BECAUSE IT TAKES A NUMBER OF YEARS TO DEVELOP PROJECTS ALL THE WAY INTO THE CLINIC. AND THE CURRENT NIH SYSTEM IS REALLY WEAK BECAUSE IT'S NOT INTEGRATED ACROSS ALL THOSE YEARS, AND THERE'S DIFFERENT STUDY SECTIONS AT DIFFERENT TIMES WHEN YOU ARE TRYING TO DEVELOP A TRIAL.

SO YOU MIGHT HAVE A STUDY SECTION THAT REVIEWS THE BASIC AND PRECLINICAL STUFF, AND A DIFFERENT STUDY

SECTION REVIEWS THE GRANTS FOR DEVELOPMENT OF THE REAGENTS AND THE TOXICOLOGY AND TOXICITY STUDIES ON THE REAGENT, AND A DIFFERENT STUDY SECTION REVIEWS THE CLINICAL TRIAL ITSELF.

AND SO IT WOULD BE EFFECTIVE, I THINK THERE SHOULD BE ONE REVIEW FOR THE WHOLE PROJECT, BUT MAYBE THEN HAVE MILESTONES OVER A NUMBER OF YEARS WHERE WHEN YOU OBTAIN A MILESTONE, THEN THERE'S A RELEASE OF FUNDING FOR THE NEXT PHASE OF THE PROJECT. SO I WOULD -- MY ADVICE WOULD BE THAT THE FUNDING BE QUITE LONG, BUT THAT THERE BE SOME PROCESS OF ADMINISTRATIVELY, NOT RE-REVIEWING, BUT AT LEAST ESTABLISHING MILESTONES THAT ARE GOING TO BE ATTAINED BEFORE RELEASE OF THE NEXT PHASE OF THE PROJECT MONEY.

DR. CHIU: RIGHT. I THINK WE --

CHAIRMAN ORKIN: I THINK THAT'S AN EXCELLENT SUGGESTION. I THINK THAT'S WHAT ARLENE MAY HAVE BEEN ALLUDING TO IN SOME RESPECTS WAS THE MILESTONES.

DR. CHIU: RIGHT. BUT FOR THE WHOLE PROJECT, HOW MANY YEARS? EVEN WITH THE MILESTONES AS STOP-GO TOUCHPOINTS AFTER THE REVIEW OF THE WHOLE PROJECT BY THE GRANTS WORKING GROUP, HOW LONG DO YOU THINK SUCH A PROJECT SHOULD BE FUNDED FOR? AND THEN TO MOVE ONTO CLINICAL TRIALS ONCE YOU GET THE IND SO THAT WE DON'T SLOW THINGS DOWN? WHAT ARE YOUR THOUGHTS?

DR. WILLIAMS: I THINK IF YOU'RE GOING TO GO ALL THE WAY TO THE IND AND ENROLLMENT IN A PHASE I TRIAL, IF THAT'S YOUR GOAL, I THINK YOU HAVE TO HAVE AT LEAST SEVEN YEARS OF FUNDING PERSONALLY.

DR. CHIU: SEVEN YEARS. OKAY. ANYBODY ELSE HAVE THOUGHTS OR EXPERIENCE?

CHAIRMAN ORKIN: I AGREE, BUT I WOULD HAVE OFFHAND SAID SOMETHING ON THE ORDER OF FIVE YEARS WITH SOME SORT OF ADMINISTRATIVE REVIEW AS TO WHETHER MILESTONES HAVE REALLY BEEN ACHIEVED, AND THEN THE RELEASE OF MONEY FOR SOME PERIOD THEREAFTER.

DR. BRIVANLOU: I AGREE WITH THAT.

DR. CSETE: THAT'S GOOD.

MR. KLEIN: SOUNDED LIKE A VERY GOOD IDEA.

DR. STEWARD: THIS IS ANOTHER QUESTION FOR CIRM. I THINK THAT THE ANSWER HAS TO BE ADDRESSED WITH RESPECT TO THE NUMBER THAT YOU PROPOSE FUNDING NOW AND THE NUMBER THAT YOU WOULD HOPE TO FUND IN THE FUTURE. IF YOU'RE FUNDING X, LET'S SAY TEN, NOW FOR SEVEN YEARS AND THERE'S NO OPPORTUNITY FOR OTHERS COMING IN, YOU RUN THE REAL RISK OF STARTING THE PROCESS AND MAYBE IN TWO YEARS OUR BASIC SCIENCE IS GOING TO BRING US TO THE POINT OF REALLY THE THRESHOLD OF ONE OF THESE THINGS, BUT NOT HAVE ANY MONEY AVAILABLE FOR IT.

SO MY QUESTION IS IS THIS A COMPREHENSIVE PLAN

1072 S.E. BRISTOL STREET, SUITE 100, SANTA ANA HEIGHTS, CALIFORNIA 92707
PHONE: 714.444.4100 FAX: 714.444.4411 EMAIL: DEPO@DEPO1.COM

FOR THE YEARS OF EXISTENCE OF CIRM, OR HOW DO YOU THINK ABOUT THIS?

DR. CHIU: I'M GLAD YOU BROUGHT THAT UP. IT WAS A POINT THAT PEOPLE AT THE WORKSHOP WERE WONDERING ABOUT AS WELL, AND I WANTED TO SEE HOW THE GRANTS WORKING GROUP FELT ABOUT IT. WE THOUGHT JUST AT THAT TIME ONE POSSIBILITY WAS TO FUND A FIRST TRANCHE WITH VERY CLEAR MILESTONES. AND IF THE MILESTONES ARE NOT MET, EVEN IF THEY ARE SUPPOSED TO BE FOR FIVE YEARS PLUS, AND I SAY FIVE YEARS PLUS BASED ON WHAT DR. ORKIN HAS JUST SAID AND ALSO IT SEEMS TO MAKE GOOD SENSE, SO THAT IT COULD BE AS LONG AS SEVEN OR EIGHT YEARS FOR THE CLINICAL TRIAL. LET'S SAY IT'S THE FIRST FIVE YEARS, BUT IF MILESTONES ARE NOT MET, IT ENDS. THE MONEY WILL BE RECYCLED FOR ANOTHER ROUND.

SO IF WE SPACE OUT ROUNDS AND WE FUND MORE TO START WITH, ASSUMING THAT THINGS WILL FAIL AND THEY WILL SHRINK AND NOT RUN TO COMPLETION BASED ON LIKELIHOOD OR UNLIKELIHOOD, AND BETTINA MENTIONED FEASIBILITY FOR ADJUSTMENT, IF IT'S A MINOR THING THAT A MILESTONE IS NOT MET, EASILY FIXABLE, THAT SHOULDN'T STOP IT. BUT IF CLEARLY NEW TECHNOLOGY HAS COME OUT IN THIS APPROACH, THEN IT SHOULDN'T CONTINUE ON OLD TECHNOLOGY AND THINGS LIKE THAT. THAT'S A POSSIBILITY.

THE OTHER IDEA IS -- AND THEN WE'LL RECYCLE

THAT APPROACH, ANOTHER DISEASE TEAM ROUND AGAIN, OR SOMETHING LIKE THAT. THAT WAY IN MY MIND WERE THESE PYRAMIDS. SO YOU FUND A LARGER NUMBER THAT SHRINKS UP TO A FEW THAT REACH THE GOAL OF A CLINICAL TRIAL. THE NEXT TRANCHE, WHICH OVERLAPS A LITTLE BIT, WILL TAKE MONEY RECYCLED BACK AND FUND MORE, AND THAT WAY WE WON'T WASTE ANY TIME IN THE LIFETIME OF CIRM.

THE SECOND THOUGHT WAS, AS DR. ORKIN SAID, IF WE FUND SOMETHING LIKE A FIVE PLUS THREE YEAR, LET'S JUST TAKE IT OFF THE TOP OF OUR HEADS, THEN AT THE END OF FIVE YEARS, IF YOU'RE READY TO GO INTO CLINICAL TRIAL, IT MAY NOT NEED A COMPREHENSIVE RE-REVIEW, BUT TO MOVE ON TO THE CLINICAL TRIAL, IF IT HASN'T REACHED THAT POINT, IT ENDS AT FIVE YEARS. THAT WAY WE DON'T SLOW THE PROGRAM, BUT YET HAVE THE FLEXIBILITY TO FUND UP TO SEVEN OR EIGHT YEARS TO GET INTO PHASE I.

HOW DOES THE WORKING GROUP FEEL ABOUT SOMETHING LIKE THIS?

CHAIRMAN ORKIN: I THINK REALISTICALLY THERE WON'T BE A LARGE NUMBER OF THESE AT THE OUTSET. I THINK IT WILL TAKE A COUPLE OF YEARS TO BUILD UP A NUMBER OF THESE PROGRAMS. IN A WAY, IT WOULD BE MORE OF A RISE AND THEN SOME SORT OF PLATEAU AS THINGS MATURE. SO I DON'T THINK IT'S AS IF YOU'D FUND THE LARGE NUMBER AT THE OUTSET AND THEN HAVE NO MONEY LEFT TO DISTRIBUTE FOR

FUTURE PROJECTS.

DR. CHIU: WE DEFINITELY HAVE TO BALANCE OR NOT SPEND EVERYTHING FOR THIS INITIATIVE WHEN NEW TECHNOLOGIES ARE COMING ON BOARD IN A YEAR, TWO, OR THREE.

DR. WILLIAMS: I GUESS THE OTHER VIEW, THOUGH, IS THAT YOU MAY HAVE, AND I MEANT TO SAY THIS BEFORE, THERE MAY BE PROJECTS THAT COME IN LATER IN THE CONTINUUM FROM THE VERY BEGINNING. IN OTHER WORDS, IT'S AT LEAST THEORETICALLY POSSIBLE THAT YOU COULD HAVE PEOPLE COMING IN WHO HAVE ALREADY DONE A LOT OF PRECLINICAL WORK AND ARE ESSENTIALLY AT A POINT WHERE WHAT THEY WANT FUNDING FOR IS ACTUAL CLINICAL TRIALS. SO I THINK THAT YOU NEED TO HAVE -- THE SYSTEM HAS TO BE FLEXIBLE ENOUGH ALSO TO ACCOMMODATE THOSE TYPES OF APPLICATIONS.

TO GET BACK TO MY POINT, WHAT I'VE SEEN THROUGH THE NIH SYSTEM IS THAT THIS CONTINUITY BETWEEN THE REVIEW OF DIFFERENT SEGMENTS REALLY SLOWS THINGS DOWN AND REALLY HINDERS THE FINAL PRODUCT, WHICH AT THE VERY LEAST SHOULD BE MAJOR POINTS GETTING INTO HUMAN TRIALS BECAUSE DURING THE TIMEFRAME THAT THE PRECLINICAL WORK IS BEING DEVELOPED, IF THERE'S ANOTHER REVIEW FOR THE CLINICAL TRIALS, WHAT OFTEN HAPPENS IS THE NEXT REVIEW GROUP DOESN'T NECESSARILY EVEN AGREE WITH THE FIRST REVIEW GROUP'S IDEAS ABOUT WHAT'S GOOD TARGETS OR WHAT'S GOOD

THIS OR THAT. AND SO THAT BECOMES KIND OF A PROBLEM BECAUSE YOU'VE INVESTED A LOT OF TIME AND A LOT OF MONEY IN DEVELOPING THE TRIAL, AND THEN SUDDENLY, BECAUSE A DIFFERENT REVIEW GROUP IS LOOKING AT IT, THERE'S A CHANGE IN THE FEELING ABOUT GOING FORWARD.

I THINK TO THE EXTENT YOU CAN DEVELOP A SYSTEM THAT REALLY LOOKS AT THE WHOLE PROJECT FROM START TO FINISH, ALBEIT, AS STU SAID, WITH THE MILESTONES BUILT IN, THAT WOULD BE MORE EFFICIENT, AND YOU WILL HAVE, I THINK, MORE PROJECTS REACH WHAT SOUNDS LIKE IS YOUR REALLY END POINT THAT YOU WANT, WHICH IS ACTUALLY TESTING IN HUMANS.

DR. CHIU: RIGHT. I ABSOLUTELY AGREE WITH YOU, AND I DON'T THINK -- REVIEW TAKES TIME, WRITING AN APPLICATION TAKES TIME, AND WE DO NOT WANT A DISEASE TEAM APPROACH TO BE BROKEN UP, AS YOU SAID, AND REVIEWED MULTIPLE TIMES. SO ONE POSSIBILITY, AFTER ALL, YOU ALL ARE THE REVIEWERS, IS THAT IF YOU APPROVE A COMPREHENSIVE PROJECT, LET'S SAY, FOR THE FIVE YEARS WITH POTENTIAL FOR MOVING INTO THE CLINIC SHOULD THEY GET AN IND, AND IF WITHIN A YEAR BEFORE THE END OF THAT IT LOOKS LIKE IT'S LIKELY, IF WE JUST DRAW BACK, SAY, THE PRIMARY AND SECONDARY REVIEWERS TO TAKE A QUICK LOOK AT PROGRESS, WE CAN MAYBE HASTEN OR SMOOTH OUT THAT TRANSITION AND MOVE STRAIGHT TO THE PLUS THREE FOR THE CLINICAL TRIALS

WITHOUT A COMPLETE RE-REVIEW.

DR. CSETE: I THINK THAT'S A GOOD IDEA.

DR. BONNER-WEIR: YEAH.

DR. CHIU: THAT'S SOMETHING WE SHOULD REALLY TAKE INTO CONSIDERATION AND PRESENT TO THE BOARD, THEN, FOR THEIR CONSIDERATION.

THIS HAS BEEN FANTASTIC. I JUST WONDER IF YOU GUYS HAVE ANY MORE IDEAS THAT WE CAN ADOPT, OR HAVE WE REALLY RUN THIS? WE REALLY APPRECIATE THIS.

CHAIRMAN ORKIN: YES.

DR. CHIU: SO IF THERE ARE NO MORE, I TURN THE MEETING BACK TO YOU.

CHAIRMAN ORKIN: IS BETTINA DONE YET? I THOUGHT THERE WERE A FEW MORE SLIDES LEFT IN THE PRESENTATION.

DR. STEFFEN: MOVING ON IS KUMAR HARI. I'M FINISHED.

CHAIRMAN ORKIN: THANK YOU VERY MUCH, BETTINA. KUMAR, I THINK YOU HAVE THE STAGE AGAIN.

DR. HARI: THANK YOU, STU. THAT'S GOING TO BE A TOUGH ACT TO FOLLOW. THAT WAS REALLY EXCELLENT FEEDBACK, AND THANK YOU FOR THAT.

HERE WE'RE ON TOPIC, I BELIEVE IT IS, SEVEN ON THE AGENDA AND NO. 5 ON YOUR SLIDE SET, WHICH IS AN UPDATE REGARDING A GRANTS ADMINISTRATION POLICY THAT CIRM

IS DEVELOPING FOR FOR-PROFIT ORGANIZATIONS.

ON THE NEXT SLIDE, WHICH IS THE PURPOSE SLIDE, WE DESCRIBE THAT THE FOR-PROFIT GRANTS ADMINISTRATION POLICY IS INTENDED TO SERVE AS THE TERMS AND CONDITIONS GOVERNING THE SELECTION, FUNDING, TERMINATION, AND MONITORING, AND THAT'S DURING AND POST AWARD, OF CIRM GRANTS.

AND THE KEY IN DEVELOPING THIS POLICY NOW IS TO OPEN THE DOOR FOR FOR-PROFIT ENTITIES TO COMPETE AGAINST ACADEMIC AND NONPROFITS FOR CIRM FUNDING BECAUSE CURRENTLY MOST RFA'S OR ALL RFA'S HAVE BEEN OPEN TO ONLY ACADEMIC AND NONPROFIT INSTITUTIONS.

THE METHOD THAT WE'VE BEEN USING THUS FAR TO DEVELOP THIS GRANTS ADMINISTRATION POLICY IS TO UTILIZE THE CURRENT AND EXISTING GAP FOR ACADEMIC AND NONPROFIT INSTITUTIONS AS A STARTING TEMPLATE AND DEFINE CHANGES THAT WOULD NEED TO BE MADE THAT ARE SPECIFIC TO FOR-PROFIT ENTITIES.

NOW, ON SEPTEMBER 7TH WE HELD AN INTERESTED PERSONS MEETING. AND THE PURPOSE OF THAT MEETING WAS TO GATHER FEEDBACK FROM FOR-PROFIT ENTITIES AS WELL AS THE PUBLIC WHERE WE REALLY ASKED PEOPLE TO FOCUS ON THOSE SITUATIONS THAT WOULD BE UNIQUE TO FOR-PROFIT ENTITIES IN TERMS OF ADMINISTERING GRANTS FROM THE CIRM.

ON THE NEXT SLIDE AND ACTUALLY THE NEXT, I

BELIEVE, THREE SLIDES, I WILL GO THROUGH FOUR TOPICS THAT WE ASKED PEOPLE TO COME AND THINK ABOUT AND DISCUSS WITH YOU.

THE FIRST TOPIC HAD TO DO WHAT UNIQUE CHANGES IN PROJECT CONTROL MIGHT ARISE IN FOR-PROFIT ORGANIZATIONS AS OPPOSED TO ACADEMIC OR NONPROFIT SETTINGS. AND FOLLOWING ON FROM THAT, HOW SHOULD THESE CHANGES BE EFFECTIVELY MANAGED BY THE FOR-PROFIT ENTITY?

(INTERRUPTION IN PROCEEDINGS.)

DR. HARI: I'LL JUST GO ONE STEP BACK, WHICH IS TO INTRODUCE THE DISCUSSION TOPICS THAT WE ASKED PEOPLE TO CONSIDER AND BRING FEEDBACK ON.

SO THE FIRST OF THESE TOPICS HAD TO DO WITH WHAT UNIQUE CHANGES IN PROJECT CONTROL MIGHT ARISE IN FOR-PROFIT ORGANIZATIONS. THAT'S AS OPPOSED TO ACADEMIC OR NONPROFIT SETTINGS. AND FOLLOWING ON FROM THAT, HOW SHOULD THESE CHANGES BE EFFECTIVELY MANAGED BY THOSE FOR-PROFIT ENTITIES AS WELL AS CIRM?

NOW, THE NOTE THAT WE TOLD PEOPLE IS THAT THE GRANTS ADMINISTRATION POLICY FOR ACADEMIC AND NONPROFIT INSTITUTIONS ALREADY REQUIRES THAT ORGANIZATIONS REQUEST PRIOR APPROVAL TO AUTHORIZE CHANGES IN PI STATUS OR AWARD TRANSFER. AND THE FEEDBACK THAT WE RECEIVED FROM THIS INTERESTED PERSONS MEETING IS THAT THE CURRENT MODEL IN REQUESTING PRIOR APPROVAL FOR CERTAIN CHANGES IS

SUFFICIENT IF THE APPROPRIATE TERMS RELATIVE TO HOW FOR-PROFIT ORGANIZATIONS DO BUSINESS ARE INCORPORATED INTO THE NEW GRANTS ADMINISTRATION POLICY. AND TWO OF THE APPROPRIATE TERMS THAT WE DISCUSSED WERE MERGERS AND ACQUISITIONS AND ALSO BANKRUPTCY.

ON THE NEXT SLIDE, DISCUSSION TOPICS 2 AND 3 ARE LISTED. TOPIC 2 HAD TO DO WITH THE CIRM'S REQUIREMENT FOR ANNUAL FINANCIAL AND PROGRAMMATIC REPORTS, AS WELL AS REPORTS OF LICENSING ACTIVITIES, PATENT APPLICATION, AND COMMERCIALIZATION ACTIVITIES FOR CIRM-FUNDED FOR-PROFIT ENTITIES.

AND THE QUESTION WE POSED IS WHAT INFORMATION CAN REASONABLY BE PROVIDED TO THE CIRM TO ACCOUNT FOR THESE REPORTS? AND THE FEEDBACK WE RECEIVED HERE WAS THAT PARTICIPANTS WOULD REALLY LIKE TO HAVE MORE CLARITY ON PRECISELY WHAT INFORMATION THE CIRM WILL NEED TO MANAGE ITS RESPONSIBILITIES TO THE STATE. AND FOR MORE BACKGROUND ON THAT, I WOULD REFER PEOPLE TO THE INTELLECTUAL PROPERTY POLICY FOR FOR-PROFIT ORGANIZATIONS TO GET A SENSE OF WHAT REPORTS WE WILL BE REQUIRING. AND INTERNALLY WE WILL THINK VERY CAREFULLY ABOUT THE TYPES OF INFORMATION THAT WE WILL REQUIRE TO, AGAIN, MANAGE OUR RESPONSIBILITIES TO THE STATE.

WITH RESPECT TO TOPIC NO. 3, WE ASKED WHAT ISSUES MAY ARISE IN SUBCONTRACTING OR PURCHASING GOODS

AND SERVICES FROM NON-CALIFORNIA ENTITIES. AND HERE THERE IS A PARTICULAR REQUIREMENT WITHIN PROPOSITION 71 THAT ALL RESEARCH FUNDING FROM THE CIRM MUST BE SPENT WITHIN THE STATE OF CALIFORNIA. YET THERE IS A PREFERENCE FOR CALIFORNIA SUPPLIERS OF GOODS AND SERVICES. AND PARTICIPANTS HERE ASKED US TO PROVIDE MORE CLEAR DEFINITIONS OF WHAT CONSTITUTES RESEARCH VERSUS GOODS AND SERVICES IN THE CONTEXT OF THE WORK THAT THEY WOULD BE PERFORMING UNDER CIRM-FUNDED GRANTS.

ON THE FINAL SLIDE, DISCUSSION TOPIC NO. 4, WE POSED THE TOPIC THAT GIVEN THAT THERE ARE VARIOUS METHODS OF ACCOUNTING FOR GRANT-RELATED ACTIVITIES IN FOR-PROFIT ORGANIZATIONS, HOW WILL FOR-PROFITS CALCULATE DIRECT RESEARCH FUNDING COSTS AND INDIRECT COSTS RELATIVE TO PROPOSITION 71 AND OUR CURRENT APPLICATIONS FOR CIRM GRANTS?

AND IT TURNS OUT THAT THE NIH INDIRECT RATES ARE THE BASIS FOR THIS TWO-PART SYSTEM THAT CIRM USES. AND MANY OF THE MEETING PARTICIPANTS ALREADY HAVE NEGOTIATED RATES, AND THEY'RE QUITE COMFORTABLE WITH THIS SYSTEM AND THEY WOULD LIKE TO STICK WITH IT. THOSE INSTITUTIONS WHO DO HAVE NIH-NEGOTIATED RATES ASSUME THAT THEY ARE GOING TO NEGOTIATE THESE RATES WITH THE CIRM. AND SO THERE ARE VARIOUS DEFINITIONS FOR WHAT CONSTITUTES INDIRECT COSTS AND DIRECT RESEARCH FUNDING THAT THE CIRM

WILL NEED TO CONSIDER.

THOSE ARE THE FOUR TOPICS AND JUST A BRIEF SUMMARY OF THE FEEDBACK THAT WE HAVE RECEIVED ON THE GRANTS ADMINISTRATION POLICY FOR FOR-PROFIT ORGANIZATIONS. OUR PATH FORWARD IS TO PREPARE A DRAFT DOCUMENT FOR PUBLIC CONSUMPTION AND THEN HOLD A SECOND INTERESTED PARTIES MEETING TO RECEIVE ADDITIONAL FEEDBACK ON THE PRECISE LANGUAGE THAT WE INCLUDE WITH THE INTENT OF PUTTING TOGETHER A VERY STRONG DRAFT TO BRING TO THE ICOC FOR APPROVAL.

AND WITH THAT, I'LL JUST OPEN IT UP FOR ANY QUESTIONS OR COMMENTS FROM THE GRANTS WORKING GROUP OR THE PUBLIC.

CHAIRMAN ORKIN: JUST ONE QUESTION IS WHAT FRACTION OF THESE FOR-PROFITS ARE LOOKING TO NEGOTIATE RATES, MEANING DON'T HAVE RATES ALREADY?

DR. HARI: JUST BASED ON THE NUMBER OF PARTICIPANTS AT THE MEETING ON SEPTEMBER 7TH, MY SENSE WAS UPWARDS OF 80 OR 90 PERCENT OF THOSE INDIVIDUALS ALREADY HAD NIH-NEGOTIATED RATES. AND I BELIEVE WE HAD REPRESENTATION FROM SOMEWHERE BETWEEN SIX AND TEN COMPANIES THERE. SO IT'S ACTUALLY A HIGH PROPORTION OF THESE INDIVIDUALS WHO ALREADY HAVE INTERACTED WITH THE NIH TO DEFINE THOSE RATES.

CHAIRMAN ORKIN: I WAS THINKING THAT IF IT WAS

THE OTHER WAY AROUND, YOU MIGHT WANT TO SET ONE RATE FOR THE FOR-PROFITS SO YOU WOULDN'T HAVE TO NEGOTIATE EACH ONE.

DR. HARI: THAT'S A VERY GOOD SUGGESTION. MY SENSE IS THAT THE MECHANISM BY WHICH THE NIH NEGOTIATES THESE INDIRECT RATES IS ACTUALLY QUITE LABOR AND RESOURCE INTENSIVE BECAUSE THEY DO SO ON AN ANNUAL BASIS. AND I BELIEVE THAT THAT'S SOMETHING WE'RE GOING TO HAVE TO TACKLE INTERNALLY HERE AT CIRM.

CHAIRMAN ORKIN: IF YOU HAD A BLANKET RATE THAT WAS SORT OF A COMPROMISE, THAT MIGHT GET YOU AWAY FROM THE ADMINISTRATIVE LOAD.

DR. CSETE: WHY DON'T YOU JUST LOOK AT THE MEANS OF THESE NEGOTIATED RATES AND SEE WHAT THAT IS.

DR. HARI: THAT'S ALSO A GOOD SUGGESTION.

DR. BRIVANLOU: I DON'T KNOW IF THIS IS THE RIGHT FORUM ON TOPIC 1 TO ADDRESS THIS FOR ACADEMIA AND INDUSTRY. MY QUESTION IS, NO. 1, IF FOR-PROFIT INSTITUTION, ARE THEY GOING TO SHARE REAGENTS AND INFORMATION WITH THE ACADEMIC SECTOR? IT'S PROBABLY THE INTELLECTUAL PROPERTY AGREEMENTS. OR ARE THEY GOING TO BE EXCLUSIVE IN THE USE OF THEIR OWN BATTERY IN EXCLUSION OF ACADEMIA? THAT'S MY FIRST QUESTION.

THE SECOND QUESTION, I'M SURE, IS ONE OF (UNINTELLIGIBLE). ARE THOSE TYPES OF INNOVATION, IF NOT

SUCCESSFUL IN RAISING FUNDS, BASED ON THE PRODUCT? WILL THEY SHARE THE PROFITS WITH THE STATE OF CALIFORNIA?

DR. HARI: YEAH. THOSE ARE TWO EXCELLENT QUESTIONS, ALI. BOTH OF THOSE QUESTIONS ARE ACTUALLY ADDRESSED IN THE INTELLECTUAL PROPERTY POLICY FOR-PROFIT ORGANIZATIONS. AND, AGAIN, I REFER PEOPLE BACK TO THAT. IT'S POSTED ON OUR WEBSITE, AND IT'S APPROACHING THE OAL FOR APPROVED REGULATION.

THE ANSWER TO THE FIRST QUESTION ABOUT SHARING REAGENTS IS WITHIN THE POLICY THERE IS A STIPULATION THAT FOR-PROFIT ORGANIZATIONS MUST SHARE MATERIALS UPON PUBLICATION, SO IT'S SIMILAR TO WHAT YOU SEE IN SORT OF ACADEMIC SETTINGS.

THE ANSWER TO THE SECOND QUESTION IS THERE'S A VERY EXTENSIVE REQUIREMENT FOR REVENUE SHARING FOR FOR-PROFIT ORGANIZATIONS THAT RECEIVE CIRM FUNDING. AND IT'S DEPENDENT -- THE STRUCTURE IS DEPENDENT UPON WHETHER A PRODUCT IS -- A CIRM-FUNDED PATENTED INVENTION IS DEVELOPED UNDER A LICENSE TO A THIRD PARTY OR BECOMES A COMMERCIALIZED PRODUCT. YOU SEE TWO DIFFERENT STRUCTURES BASED ON WHAT PATH A CIRM-FUNDED ORGANIZATION TAKES.

DR. BRIVANLOU: KUMAR, I'M WORRIED ABOUT THE THIRD TOPIC. SHARING REAGENTS DOWNSTREAM OF PUBLICATION MIGHT NOT BE VERY USEFUL FOR COLLEAGUES IN ACADEMIA BECAUSE ONE PUBLICATION IN THE PRIVATE SECTOR, AS WE

KNOW, IS QUITE DIFFERENT THAN THE REQUIREMENT OF PUBLICATION IN ACADEMIA. IF THE COMPANY DECIDES NOT TO PUBLISH THEIR INFORMATION, THEY CAN CONTINUE WORKING WITHOUT SHARING REAGENTS. I WAS JUST WONDERING IN THE SPIRIT OF CIRM AND WHAT WE'RE TRYING TO ACCOMPLISH HERE, CAN WE HAVE A SPECIAL CASE FOR HUMAN EMBRYONIC STEM CELLS AT LEAST TO SEE IF ANY INNOVATIONS THAT ARE DEVELOPED FUNDED WITH CIRM WOULD AT LEAST BE SHARED AMONG THE CIRM ACADEMIC MEMBERS?

DR. HARI: THUS FAR, AND I DON'T SEE SCOTT TOCHER IN THE ROOM, WHO IS THE PERSON WHO HAS BEEN MOST INVOLVED IN PREPARING INTELLECTUAL PROPERTY POLICY FOR FOR-PROFIT ORGANIZATIONS. THUS FAR, THAT POLICY DOESN'T CONTEMPLATE DIFFERENT STRUCTURES FOR SHARING REAGENTS WITH ACADEMICS VERSUS OTHER FOR-PROFIT ORGANIZATIONS, FOR EXAMPLE, BUT WE'D CERTAINLY TAKE THAT FEEDBACK AND WILL SHARE THAT WITH SCOTT TOCHER SO THAT HE MIGHT CONSIDER INCORPORATING SOME MECHANISM WITHIN THAT POLICY.

MR. KLEIN: KUMAR, IS THERE SOMEONE THAT COULD JUST ASK IF SCOTT COULD COME IN TO ANSWER THESE QUESTIONS BECAUSE THEY ARE VERY IMPORTANT? AND SCOTT SHOULD BE VERY CLOSE PHYSICALLY TO WHERE YOU ARE HOLDING YOUR MEETING.

DR. HARI: IN FACT, HE IS STANDING WITHIN 5 FEET OF ME NOW. ARLENE JUST WENT AND GRABBED HIM, SO,

ALI, IF I COULD ASK YOU MAYBE TO REPEAT YOUR QUESTIONS FOR SCOTT TOCHER, WHO'S NOW HERE.

DR. CSETE: ALI, MAYBE YOU WOULD BE SATISFIED IF IT WAS UPON PUBLICATION OR COMMERCIALIZATION?

DR. BRIVANLOU: I WOULD BE SATISFIED FROM DISCOVERY, TO BE HONEST WITH YOU, BECAUSE I THINK THIS IS ONE OF THOSE AREAS WHERE WE CAN ALL AGREE THAT WHAT IS PERFECTLY FINE TO COMPETE IN THE FREE MARKET THAT IS ALREADY MENTIONED THAT WE WANT TO PUT FORWARD. I REALLY DON'T HAVE THE ANSWER TO THAT. MY EXPERIENCE IN THE PAST, INCLUDING WITH COMPANIES IN CALIFORNIA WHO ARE LEADERS IN THE STEM CELL FIELD, IS THAT SHARING OF REAGENTS WAS NOT AUTOMATIC AT ALL. AND, IN FACT, IT TOOK A LONG, LONG TIME TO GET REAGENTS. AND IT TOOK A LOT OF US IN OTHER PLACES TO GO AHEAD AND DERIVE OUR OWN JUST BECAUSE OF THAT LACK OF SHARING.

TO REPEAT THE QUESTION, MY FIRST QUESTION IS WHAT IS THE RULES OF SHARING REAGENTS BETWEEN THE FOR-PROFIT ORGANIZATIONS AND THE SCIENTISTS WORKING IN ACADEMIA? AT A MINIMUM WOULD IT BE A DIRECT EXCHANGE OF INFORMATION WITHOUT ANYTHING ATTACHED BETWEEN THE CIRM-FUNDED ACADEMIC RESEARCHERS AND THE CIRM-FUNDED FOR-PROFIT RESEARCHERS?

MR. TOCHER: BOTH FOR- AND NONPROFIT POLICIES ARE, AND FORGIVE ME IF THIS HAS ALREADY BEEN DISCUSSED,

BUT THEY'RE KEYED OFF OF PUBLICATION. SO ABSENT THAT TRIGGER, IT WOULD JUST OCCUR IN THE NORMAL COURSE THE WAY THINGS HAPPEN IN THE NORMAL COURSE OF BUSINESS BETWEEN RESEARCHERS. AND SO I'M NOT REALLY SURE THAT OUR POLICY, THEN, REALLY DIFFERENTIATES FROM CURRENT PRACTICE.

MR. KLEIN: THE THRUST OF HIS POINT WAS THAT FOR-PROFITS COULD CHOOSE NOT TO PUBLISH OR PUBLISH FOR AN EXTENDED PERIOD OF TIME. SO IS THERE A MECHANISM OR HAVE YOU DISCUSSED THE ISSUE OF A TIME PERIOD AFTER DISCOVERY OR SOME OTHER TRIGGERS BECAUSE EARLIER ON THERE WAS DISCUSSION, I KNOW, OF IF THEY MADE A PRESENTATION TO A SCIENTIFIC CONFERENCE, THERE WAS AN ISSUE WHETHER OF YOU COULD TRIGGER OFF OF THAT IN THE ABSENCE OF PUBLICATION.

MR. TOCHER: I THINK THAT THE -- AS YOU KNOW, THIS WAS SHAPED INITIALLY IN THE CONTEXT OF THE DEVELOPMENT OF A NONPROFIT POLICY. AND SO I THINK THE CONCLUSION WAS TO JUST TRACK CURRENT PRACTICE OF MOST JOURNALS. WHEN THESE THINGS PUBLISHED, THIS IS THE SORT OF EXPECTATION OF PEOPLE WHO PUBLISH. SO BEYOND THAT, I DON'T THINK ANY OTHER TRIGGERS WERE EXPLORED, CERTAINLY NOT IN THE FOR-PROFIT CONTEXT.

DR. BRIVANLOU: THEN I RESPECTFULLY SUGGEST THAT THIS BE PUT AS A PRIORITY BECAUSE I'M SURE WE DON'T WANT TO CREATE THE SITUATION WHERE CIRM-FUNDED PEOPLE COMPETE AGAINST ONE ANOTHER IN THE PRIVATE SECTOR VERSUS

ACADEMIA AND SOME OF THE INFORMATION FROM ONE SIDE AND NOT FROM THE OTHER SIDE.

DR. CHIU: I THINK YOU BRING UP A VERY GOOD POINT, THAT DELAY OF PUBLICATION COULD PREVENT REAGENTS FROM BEING SHARED AS FREELY AS POSSIBLE. AND AT THE CURRENT STATE, I'M NOT EXACTLY SURE WHERE WE ARE; BUT WHEN THESE REGULATIONS WERE OPEN FOR PUBLIC COMMENT, THAT MIGHT HAVE BEEN SOMETHING TO HAVE BEEN ADDRESSED. LET'S SEE WHAT WE CAN DO TO MOVE THIS FORWARD. I'M NOT SURE WHERE IN THE PROCESS WE ARE RIGHT NOW, BUT WE WILL CERTAINLY TAKE YOUR POINT.

MR. TOCHER: I CAN ELABORATE A LITTLE BIT ON THE PROCESS. WE'RE SORT OF WINDING DOWN FINE-TUNING THE FOR-PROFIT POLICY. AND THERE IS NOT ANTICIPATED TO BE ANOTHER MEETING OF THE TASK FORCE PRIOR TO BRINGING IT, I THINK, TO THE ICOC. BUT, OF COURSE, WE CAN ALWAYS TALK ABOUT THIS AT THE VERY LEAST WITH ED AND MARY AND GET THEM ON THE SCREEN ON IT, AND IT WILL CERTAINLY COME BEFORE THE ICOC BEFORE IT GOES OFF TO OAL AND BECOMES FINALIZED.

DR. HARI: FOR THE BACKGROUND OF THE GRANTS WORKING GROUP, ED AND MARY IS ED PENHOET, WHO IS THE VICE CHAIR OF THE ICOC, AND MARY MAXON, WHO SOME OF YOU MAY REMEMBER, FORMER DEPUTY TO THE VICE CHAIR, WAS ALSO ON THE SCIENTIFIC STAFF.

DR. MURPHY: I THINK WHAT WE MIGHT WANT TO DO, KUMAR, BEFORE WE MOVE MUCH FURTHER IS LOOK AT THREE OR FOUR ALTERNATIVES AS TO WHAT THAT PROCEDURE MAY BE, WHAT THAT POLICY SHOULD BE, SO THAT WE HAVE AN OPPORTUNITY FOR BUSINESS TO COMMENT ON, NO. 1, THE MAJOR ISSUE, WHICH I THINK IS A VERY IMPORTANT ONE AND, NO. 2, WHAT ARE THE ALTERNATIVES THAT THEY COULD LIVE WITH AS OPPOSED TO NOT.

MR. KLEIN: THE OTHER QUESTION THAT WAS ASKED BEFORE YOU CAME IN THE ROOM, SCOTT, I THINK ALSO BY ALI, PERHAPS YOU COULD SUMMARIZE WHAT THE PAYBACK TO THE STATE PROVISIONS STIPULATE IN TERMS OF FOR-PROFIT SHARING THEIR REVENUES WITH THE STATE. AND YOU MIGHT ALSO CUT OUT AND DESCRIBE THE SPECIAL PROVISIONS THAT ADDRESS TOOLS.

MR. TOCHER: OKAY. WELL, THE REVENUE SHARING IS SORT OF KEYED OFF OF TWO SORT OF BRANCHES. EITHER THE GRANTEE WILL SORT OF SELF-DEVELOP A PRODUCT OR WILL LICENSE A CIRM-FUNDED PATENTED INVENTION. SO TAKING THE LATTER FIRST, IF A GRANTEE LICENSES AN INVENTION AND THAT PATENTED INVENTION RETURNS REVENUES TO THE GRANTEE, THE STATE, AFTER A $500,000 THRESHOLD, WILL RECEIVE 25 PERCENT OF THOSE REVENUES. AND THEN THERE'S A FORMULA WHICH ENTERS INTO THE PICTURE IF CIRM WAS NOT THE ONLY SOURCE OF FUNDS FOR THAT PATENTED INVENTION.

IT'S A LITTLE TRICKIER IF THEY SELF-DEVELOP, BUT WE'RE TRYING TO ENCOURAGE SELF-DEVELOPMENT. SO

THERE, AGAIN, AFTER A $500,000 THRESHOLD, AND HERE WE'RE NOT JUST TALKING ABOUT PATENTED INVENTIONS BECAUSE CIRM FUNDING COULD COME AT VARIOUS POINTS IN THE DEVELOPMENT OF A PRODUCT THAT IS COMMERCIALIZED, BUT ONCE THE REVENUES START COMING IN, IT'S CAPPED AT THREE TIMES CIRM'S INVESTMENT. SO OBVIOUSLY IF WE PUT IN A MILLION DOLLARS, WE'RE GOING TO GET BACK THREE.

IF THERE IS A -- HOWEVER, IF IT ACHIEVES MORE THAN $250 MILLION IN REVENUE, THAT COMMERCIALIZED PRODUCT, THEN THE STATE WILL GET ANOTHER THREE TIMES. AND THEN IF IT HITS A $500 MILLION MARK, THE STATE WILL GET A FINAL THREE TIMES AMOUNT, SO $9 MILLION ON THE $1 MILLION.

NOW, THERE'S AN EXTRA COMPONENT THERE, THAT IF THERE'S A CIRM-FUNDED PATENTED INVENTION, THEN WHEN YOU HIT THAT BLOCKBUSTER STATUS AT 500 MILLION, THERE WILL BE AN ONGOING 1-PERCENT ROYALTY PAID TO THE STATE THEREAFTER.

MR. KLEIN: IN ADDITION TO THE NINE TIMES PAYBACK.

MR. TOCHER: THAT'S RIGHT. AND THEN THERE ARE OTHER COMPONENTS. THAT'S THE FINANCIAL RETURN. I'M SORRY, BOB, I'M FORGETTING THE OTHER PART OF YOUR QUESTION.

MR. KLEIN: THE SPECIAL TREATMENT OF TOOLS IN

TERMS OF THE RESEARCH COMMONS AVAILABILITY.

MR. TOCHER: I'M SORRY. IN TERMS OF THE RESEARCH --

MR. KLEIN: COMMONS AVAILABILITY. WITH TOOLS YOU HAVE A CARVE-OUT FOR THE OBLIGATION TO PROVIDE THESE TO OTHER RESEARCH INSTITUTIONS IN CALIFORNIA.

MR. TOCHER: THAT'S RIGHT, YES. THAT HAS TO BE SHARED AMONG OTHER CIRM-FUNDED RESEARCHERS THAT ARE IN THE STATE. I WAS TRYING TO THINK OF A REVENUE COMPONENT THERE.

DR. CHIU: IT ADDRESSES DR. BRIVANLOU'S QUESTION ABOUT SHARING RESOURCES AMONG CIRM-FUNDED GRANTEES.

MR. TOCHER: RIGHT.

DR. BRIVANLOU: THANK YOU.

DR. SAMBRANO: ANY OTHER QUESTIONS? DR. ORKIN, I GUESS WE TURN IT BACK TO YOU.

CHAIRMAN ORKIN: ARE THERE ANY COMMENTS FROM THE PUBLIC? I THINK THAT'S OUR LAST BUSINESS.

MR. VALENCIA: GOOD AFTERNOON. MY NAME IS JOHN VALENCIA. I'M HERE REPRESENTING INVITROGEN CORPORATION AND ALSO APPEARING ON BEHALF OF THE CALIFORNIA HEALTHCARE INSTITUTE. BOTH ORGANIZATIONS AND COMPANY HAVE PARTICIPATED IN THE STAKEHOLDERS DISCUSSION THAT WAS REFERRED TO BY KUMAR. AND I JUST WANT TO REVISIT BRIEFLY

IN THE CONTEXT OF TOPIC NO. 2 AND NO. 3 A MORE IMMEDIATE AND DEFINITE RETURN ON INVESTMENT FROM CIRM EXPENDITURES, PROP 71 EXPENDITURES.

THE FORMULA, AND THAT WAS AN EXCELLENT INQUIRY BECAUSE THE FORMULA DESCRIBES THE SPECULATIVE POTENTIAL RETURN TO THE VOTERS AND TO THE PEOPLE OF CALIFORNIA. BOTH CHI AND INVITROGEN, A GLOBAL LEADER IN THE DEVELOPMENT OF RESEARCH TOOLS, FEELS STRONGLY THAT MORE PRECISE DEFINITION IS NECESSARY TO IMPLEMENT A PARTICULAR CODE THAT'S NOW PART OF THE HEALTH AND SAFETY CODE AFTER THE VOTER ENACTMENT OF PROP 71, ESPECIALLY IN LIGHT OF THE VOTER PURPOSE, PROBABLY, AT LEAST IN MY ESTIMATION, AFTER THE CORE PURPOSE OF STIMULATING STEM CELL RESEARCH, STIMULATING THE STATE'S ECONOMY WAS RIGHT THERE AS 1A OR PERHAPS PURPOSE NO. 2.

THE VOTERS STATED THAT A PRIME PURPOSE OF PROP 71 WAS TO BENEFIT THE CALIFORNIA ECONOMY BY CREATING PROJECTS, JOBS, AND THERAPIES THAT WILL GENERATE MILLIONS OF DOLLARS IN NEW TAX REVENUES FOR THE STATE, ADVANCE THE BIOTECH INDUSTRY IN CALIFORNIA TO WORLD LEADERSHIP AS AN ECONOMIC ENGINE FOR THE STATE'S FUTURE, AND, FINALLY, TO PROTECT AND BENEFIT THE CALIFORNIA BUDGET.

THE ORGANIZATION, THE AGENCY, HAS A PARTICULAR CHARGE IN OVERSIGHT, AND THIS WILL BE AN ADMINISTRATIVE CHALLENGE THAT WE THINK DEFINITENESS WILL HELP THE AGENCY

FULFILL. AND THAT'S ENSURING THAT ITS GRANTEES PURCHASE GOODS AND SERVICES, A TERM THAT TOPIC 3 IDENTIFIES AS ONE IN NEED OF DEFINITION AS OPPOSED TO WHAT MAY CONSTITUTE RESEARCH, THAT THE GRANTEES PURCHASE GOODS AND SERVICES FROM CALIFORNIA SUPPLIERS, A TERM ALSO IN NEED OF DEFINITION WITHIN THE POLICY TO ULTIMATELY BE ADOPTED BY THE AGENCY. ONE, BY THE WAY, FOR WHICH WE THINK THERE'S A READILY AND NON-WHEEL REINVENTING BASIS ON WHICH TO DRAW, AND THAT'S CALIFORNIA TAX LAW. IT'S VERY WELL SETTLED IN MANY RESPECTS WHAT IS AND WHAT IS NOT A CALIFORNIA ENTITY. AND IF EVER THERE WAS A BASIS THAT'S BEEN WELL THOUGHT OUT AND WELL WORKED OVER, IT'S TAX LAW SINCE THE BATTLES HAVE BEEN MANY AND THOROUGH IN TERMS OF YOU WHO PAYS WHAT AND UNDER WHAT CIRCUMSTANCES.

FINALLY, TO ENSURE THAT THE GRANTEES IN GOOD FAITH AND TO THE EXTENT REASONABLY NECESSARY ACHIEVE A SPECIFIC GOAL, AND THAT'S 50 PERCENT OF THEIR PURCHASES FROM CALIFORNIA SUPPLIERS. SO YOU CAN SEE THAT THE STATUTE NOW IN PLACE IS IN NEED OF SEVERAL GAP FILLER AND MORE PRECISE DEFINITIONS SO THAT THE FLOW LEADING BACK TO THE AGENCY'S OVERSIGHT CHARGE CAN BE READILY HANDLED. THOSE ARE OUR OBSERVATIONS AND CONTRIBUTIONS TODAY. THANK YOU.

CHAIRMAN ORKIN: THANK YOU FOR YOUR COMMENTS. ADDITIONAL COMMENTS FROM THE PUBLIC OR FROM THE

COMMITTEE? ANYONE ELSE?

DR. MURPHY: STU, RICH MURPHY. ONLY TO THANK THE WHOLE COMMITTEE FOR INVALUABLE INPUT INTO ALL OF THESE DISCUSSIONS. YOU'RE HELPING US REALLY FORM AND DEFINE THE FUTURE OF THE INSTITUTION AND OUR FUNDING, AND IT'S VERY MUCH APPRECIATED.

CHAIRMAN ORKIN: THANK YOU.

MR. KLEIN: STU, THIS IS BOB. IN TERMS OF THE LAST COMMENT, I'D LIKE TO EMPHASIZE THAT 50 PERCENT OF PURCHASES FROM CALIFORNIA SUPPLIERS IS A GOAL. IT'S NOT MEANT TO BE A GOAL THAT SUBORDINATES THIS OBJECTIVE TO THE PRIMARY MISSION, WHICH IS TO ADVANCE MEDICAL SCIENCE. SO TO THE EXTENT THAT THE SCIENTIFIC EXPERIMENTS REQUIRE THE HIGHER PERCENTAGE OF THE SUPPLIES BE OUT-OF-STATE TO ACCOMPLISH THE SCIENTIFIC MISSION, WHICH HAS TO BE DOMINANT, THAT'S THE OBJECTIVE THAT IS PRIMARY. THIS IS A SUBORDINATE GOAL WHICH HAS TO REMAIN PROPERLY IN CONTEXT.

CHAIRMAN ORKIN: THANK YOU. I THINK WE'RE PROBABLY DONE FOR THIS AFTERNOON. I'D LIKE TO THANK EVERYONE, AND I THINK WE ARE ADJOURNED.

DR. CHIU: THANK YOU SO MUCH, STU AND EVERYBODY WHO CALLED IN.

(THE MEETING WAS THEN ADJOURNED.)

1072 S.E. BRISTOL STREET, SUITE 100, SANTA ANA HEIGHTS, CALIFORNIA 92707
PHONE: 714.444.4100 FAX: 714.444.4411 EMAIL: DEPO@DEPO1.COM

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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 SCIENTIFIC AND MEDICAL GRANTS WORKING GROUP OF 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

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SEPTEMBER 19, 2007

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BETH C. DRAIN, CSR 7152
BARRISTER'S REPORTING SERVICE
1072 S.E. BRISTOL STREET
SUITE 100
SANTA ANA HEIGHTS, CALIFORNIA
(714) 444-4100