BEFORE THE

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

REGULAR MEETING

LOCATION: CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE
1999 HARRISON STREET, SUITE 1650
OAKLAND, CALIFORNIA

DATE: SEPTEMBER 28, 2017
9 A.M.

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

FILE NO.: 2017-20

I N D E X

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I N D E X (CONT'D.)

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OAKLAND, CALIFORNIA; SEPTEMBER 28, 2017

9 A.M.

CHAIRMAN THOMAS: GOOD MORNING, EVERYBODY. WE WOULD LIKE TO CALL THIS MORNING'S MEETING OF THE ICOC AND THE APPLICATION REVIEW SUBCOMMITTEE TO ORDER. BEAUTIFUL DAY IN OAKLAND. WE LOOK FORWARD TO QUITE A MEATY AGENDA, LOT OF VERY INTERESTING TOPICS. SO, MARIA, WOULD YOU LEAD US IN THE PLEDGE OF ALLEGIANCE.

(THE PLEDGE OF ALLEGIANCE.)

CHAIRMAN THOMAS: MARIA, WILL YOU PLEASE CALL THE ROLL.

MS. BONNEVILLE: GEORGE BLUMENTHAL.

DR. BLUMENTHAL: HERE.

MS. BONNEVILLE: LINDA BOXER.

DR. BOXER: HERE.

MS. BONNEVILLE: KEN BURTIS.

DR. BURTIS: PRESENT.

MS. BONNEVILLE: DEBORAH DEAS.

DR. DEAS: HERE.

MS. BONNEVILLE: JACK DIXON.

DR. DIXON: HERE.

MS. BONNEVILLE: ANNE-MARIE DULIEGE. HOWARD FEDEROFF. JUDY GASSON.

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DR. GASSON: HERE.
MS. BONNEVILLE: DAVID HIGGINS.
DR. HIGGINS: HERE.
MS. BONNEVILLE: STEPHEN JUELSGAARD. SHERRY LANSING. BERT LUBIN.
DR. LUBIN: HERE.
MS. BONNEVILLE: LINDA MALKAS.
DR. MALKAS: HERE.
MS. BONNEVILLE: DAVE MARTIN.
DR. MARTIN: HERE.
MS. BONNEVILLE: SHLOMO MELMED.
DR. MELMED: HERE.
MS. BONNEVILLE: LAUREN MILLER. ADRIANA PADILLA.
DR. PADILLA: HERE.
MS. BONNEVILLE: JOE PANETTA.
MR. PANETTA: HERE.
MS. BONNEVILLE: FRANCISCO PRIETO.
DR. PRIETO: HERE.
MS. BONNEVILLE: ROBERT QUINT. AL ROWLETT. JEFF SHEEHY.
SUPERVISOR SHEEHY: HERE.
MS. BONNEVILLE: OSWALD STEWARD.
DR. STEWARD: HERE.
MS. BONNEVILLE: JONATHAN THOMAS.

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CHAIRMAN THOMAS: HERE.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: HERE.
MS. BONNEVILLE: KRISTINA VUORI.
DR. VUORI: HERE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: HERE.
MS. BONNEVILLE: ARE THERE ANY MEMBERS OF THE PUBLIC AT THE OFFSITE LOCATIONS?
DR. DIXON: NONE IN SAN DIEGO.
DR. MELMED: NONE IN LOS ANGELES.
MS. BONNEVILLE: THANK YOU.
CHAIRMAN THOMAS: BEFORE WE START HERE, A FEW MICROPHONE ANNOUNCEMENTS. ONE IS THESE ARE PUSH-TO-TALK MICS, NO. 1. NO. 2, WHEN YOU'RE NOT TALKING, SO AS TO PRECLUDE STATIC, IF YOU COULD MAKE SURE YOUR MICS ARE OFF. FOR THOSE OF YOU ON THE PHONE, IF YOU COULD KEEP IT ON MUTE EXCEPT OBVIOUSLY WHEN YOU ARE SPEAKING, WE WOULD APPRECIATE IT. THANK YOU.
BEFORE WE GET TO THE AGENDA, I WANTED TO INTRODUCE TO YOU, WE HAVE A NEW BOARD MEMBER HERE TODAY, DAVE MARTIN, TO MY RIGHT. WE ARE DELIGHTED TO HAVE HIM ABOARD, AND I'VE ASKED HIM TO SAY A FEW WORDS OF INTRODUCTION.

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DR. MARTIN: THANK YOU, J.T. SO I'M TRAINED AS A PHYSICIAN AT DUKE. DID A RESIDENCY THERE. WENT TO NIH WEARING A YELLOW BERET TO AVOID THE VIETNAM WAR AND THEN MOVED TO UCSF WHERE OVER 13 YEARS BECAME A PROFESSOR OF MEDICINE AND BIOCHEMISTRY AND A HOWARD HUGHES MEDICAL INSTITUTE INVESTIGATOR. MOVED FROM THERE TO GENENTECH AS THE FIRST HEAD OF R&D, LEFT THERE WHEN ROCHE BOUGHT IT THE FIRST TIME, AND WENT TO DUPONT MERCK AS HEAD OF R&D. MOVED BACK TO CHIRON, WHERE THERE ARE A NUMBER OF PEOPLE THAT I KNOW FROM THERE WHO ARE HERE. THEN I STARTED A COUPLE OF BIOTECH COMPANIES. THE MOST RECENT ONE IS ONE CALLED AVIDBIOTICS IN SOUTH SAN FRANCISCO WHERE I'M THE CHAIRMAN AND CEO.

WHEN I WAS AT UCSF, ALL OF MY COLLEAGUES IN THE DEPARTMENT OF MEDICINE THOUGHT I WAS A REALLY GOOD BIOCHEMIST, AND ALL OF MY COLLEAGUES IN THE DEPARTMENT OF BIOCHEMISTRY THOUGHT I WAS A REALLY GOOD PHYSICIAN. I JUST NEVER LET THEM TALK.

CHAIRMAN THOMAS: THANK YOU, DAVE, VERY MUCH AND WELCOME TO THE BOARD.

WE'RE GOING TO GO ON TO THE ACTION ITEMS. THE FIRST ITEM IS A CLOSED SESSION. SCOTT, WILL YOU READ THE APPROPRIATE LANGUAGE?

MR. TOCHER: THANK YOU, J.T. WE WILL BE

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CONVENING IN CLOSED SESSION FOR A DISCUSSION OF PERSONNEL PURSUANT TO GOVERNMENT CODE SECTION 11126(A) AS WELL AS HEALTH AND SAFETY CODE SECTION 125290.30(F)(3)(D).

CHAIRMAN THOMAS: WELL SAID. THANK YOU. SO --

MS. BONNEVILLE: FOR THOSE OF YOU ON THE PHONE, AMY SENT YOU THE CLOSED SESSION DIAL-IN NUMBER YESTERDAY. IF YOU NEED IT, JUST PING AMY AND MYSELF AND WE WILL SEND IT TO YOU. FOR THOSE OF YOU IN THE ROOM, THE CLOSED SESSION IS THAT WAY. YOU CAN FOLLOW AMY. THANK YOU.

(CLOSED SESSION.)

CHAIRMAN THOMAS: OKAY. WE'VE NOW RECONVENED IN OPEN SESSION. WE'RE MOVING ON. FIRST ACTION ITEM IS CONSIDERATION OF APPOINTMENT OF NEW PRESIDENT INCLUDING THE COMPENSATION PACKAGE. AND IT IS WITH A GREAT DEAL OF ENTHUSIASM THAT I WOULD LIKE TO ENTERTAIN A MOTION TO APPOINT DR. MARIA MILLAN AS PRESIDENT AND CEO OF CIRM AT THE ANNUAL SALARY OF 550,000 TO BE MADE EFFECTIVE RETROACTIVE TO JULY 1ST, 2017. DO I HEAR A MOTION TO THAT EFFECT?

MS. WINOKUR: SO MOVED.

CHAIRMAN THOMAS: MOVED BY DIANE WINOKUR.

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IS THERE A SECOND?

DR. JUELSGAARD: SECOND.

CHAIRMAN THOMAS: SECONDED BY MANY PEOPLE, BUT I THINK MR. JUELSGAARD HAD HIS HAND UP FIRST, BUT THANK YOU. SO IS THERE ANY DISCUSSION BY MEMBERS OF THE BOARD ON THIS TOPIC?

I WOULD LIKE TO SAY THAT WE GAVE, AS YOU ALL KNOW, A GREAT DEAL OF CONSIDERATION TO THIS. THIS IS ONE OF THE CENTRAL MOVES THAT WE MAKE AS A BOARD, WHICH IS TO GET THE RIGHT LEADER FOR THE RIGHT TIME. AND AT THE TIME THAT RANDY ANNOUNCED HE WAS LEAVING CIRM, HE AND I BOTH STRONGLY RECOMMENDED AS OUR OVERWHELMING CHOICE FOR THEN INTERIM CEO DR. MILLAN BASED ON HER CAREER, NOT ONLY IN MANY DIFFERENT ASPECTS, WHETHER IT WAS ACADEMIA, AS A SURGEON, AS IN INDUSTRY OR WHATEVER, BUT JUST AS IMPORTANTLY THE FACT THAT SHE HAD BEEN REALLY RANDY'S RIGHT-HAND PERSON FOR AT THAT POINT ABOUT FOUR AND A HALF YEARS AND WAS INTEGRALLY INVOLVED WITH ALL ASPECTS OF CIRM, INCLUDING THE DEVELOPMENT OF THE STRATEGIC PLAN, HEADING UP THE THERAPEUTIC EFFORT, WHICH HAD THE CHARGE, VERY BOLD CHARGE, OF TRYING TO GET 50 CLINICAL TRIALS FUNDED AT CIRM BY THE YEAR 2020.

SHE HAD A SAY IN VIRTUALLY EVERY MAJOR

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DECISION WHEN RANDY WAS HERE, A VERY, VERY IMPORTANT VOICE, AND HAD DONE A GREAT JOB. AND WE FELT THAT SHE WAS ABSOLUTELY THE RIGHT PERSON FOR THIS SPOT. AS I JUST SAID TO THE BOARD IN CLOSED SESSION, HAVING SEEN MARIA AS THE INTERIM CEO FOR THREE MONTHS AND OBSERVING HER AND HOW SHE HAS OPERATED WITH THAT POSITION, I AM DOUBLING DOWN AND EVEN MORE ENTHUSIASTIC THAN I WAS BEFORE AND VERY GRATEFUL ON BEHALF OF THE ORGANIZATION THAT WE HAVE SOMEBODY OF DR. MILLAN'S CALIBER AND INTEREST, ENTHUSIASM, LEADERSHIP QUALITY, I COULD GO ON AND ON.

SO IF ANYBODY WOULD LIKE TO SAY ANYTHING ELSE. IF NOT, WE'VE HAD A LOT OF DISCUSSION ON THIS TOPIC. I WOULD PROCEED TO A ROLL CALL VOTE. MARIA, WILL YOU CALL THE ROLL. OR, SCOTT, WILL YOU CALL THE ROLL. IS THERE ANYBODY IN THE PUBLIC WHO WOULD LIKE TO SAY ANYTHING ON THIS AGENDA TOPIC? I SEE SOME CLAPPING MOTIONS IN THE AUDIENCE.

(APPLAUSE.)

MS. BONNEVILLE: GEORGE BLUMENTHAL.

DR. BLUMENTHAL: YES.

MS. BONNEVILLE: LINDA BOXER.

DR. BOXER: YES.

MS. BONNEVILLE: KEN BURTIS.

DR. BURTIS: YES.

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MS. BONNEVILLE: DEBORAH DEAS.
DR. DEAS: YES.
MS. BONNEVILLE: JACK DIXON.
DR. DIXON: YES.
MS. BONNEVILLE: ANNE-MARIE DULIEGE. HOWARD FEDEROFF. JUDY GASSON.
DR. GASSON: YES.
MS. BONNEVILLE: DAVID HIGGINS.
DR. HIGGINS: YES.
MS. BONNEVILLE: STEPHEN JUELSGAARD.
MR. JUELSGAARD: YES.
MS. BONNEVILLE: SHERRY LANSING. BERT LUBIN.
DR. LUBIN: YES.
MS. BONNEVILLE: LINDA MALKAS.
DR. MALKAS: YES.
MS. BONNEVILLE: DAVE MARTIN.
DR. MARTIN: YES.
MS. BONNEVILLE: SHLOMO MELMED.
DR. MELMED: YES.
MS. BONNEVILLE: LAUREN MILLER. ADRIANA PADILLA.
DR. PADILLA: YES.
MS. BONNEVILLE: JOE PANETTA.
MR. PANETTA: YES.

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MS. BONNEVILLE: FRANCISCO PRIETO.
DR. PRIETO: AYE.
MS. BONNEVILLE: ROBERT QUINT.
DR. QUINT: YES.
MS. BONNEVILLE: AL ROWLETT. JEFF SHEEHY.
SUPERVISOR SHEEHY: YES.
MS. BONNEVILLE: OSWALD STEWARD.
DR. STEWARD: YES.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: KRISTINA VUORI.
DR. VUORI: YES.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MS. BONNEVILLE: THE MOTION CARRIES.
CHAIRMAN THOMAS: THAT'S ABOUT AS UNANIMOUS AS IT'S GOING TO GET. CONGRATULATIONS, MARIA.
(APPLAUSE.)
CHAIRMAN THOMAS: MARIA WOULD LIKE TO ADDRESS THE BOARD HERE, FOR THOSE ON THE PHONE.
DR. MILLAN: CHAIRMAN THOMAS, MEMBERS OF THE BOARD, AND MEMBERS OF THE PUBLIC, AND

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COLLEAGUES, THANK YOU VERY MUCH FOR THIS HONOR OF SERVING AS CIRM'S NEXT PRESIDENT AND CEO. IT HAS BEEN AN ABSOLUTE PLEASURE TO BE AT CIRM AND TO WATCH THE PROGRESS THAT WE'VE MADE. AND OVER THE PAST THREE MONTHS, SERVING AS INTERIM CEO, THAT'S EVEN VALIDATED THAT NOTION EVEN MORE WATCHING FROM THAT VANTAGE POINT.

I WANT TO THANK IN PARTICULAR MY COLLEAGUES ON THE LEADERSHIP TEAM AS WELL AS THE CIRM TEAM. SINCE THE TRANSITION AND DURING THE INTERIM PERIOD, THIS GROUP, THIS TEAM, DID NOT MISS A STEP, DID NOT SKIP A BEAT, CONTINUED TO FOCUS, REMAIN 100 PERCENT COMMITTED TO THE MISSION, AND CONTINUED TO DELIVER AND, IN FACT, OVERDELIVER. AND I WILL JUST KIND OF SUMMARIZE FOR YOU SOME BRIEF EXAMPLES OF WHAT THIS TEAM HAS DONE AND CONTINUES TO DO.

THERE WILL BE A MORE COMPLETE REPORT ON OUR PERFORMANCE AT THE DECEMBER BOARD MEETING ALONG WITH A BUDGET PROPOSAL.

AND JUST PUTTING UP ON THE SLIDE HERE IS OUR MISSION. IT HAS ALWAYS BEEN OUR MISSION, IT CONTINUES TO BE OUR MISSION, AND IS MY PERSONAL MISSION, TO ACCELERATE STEM CELL TREATMENTS TO PATIENTS WITH UNMET MEDICAL NEEDS. WE'RE

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THREE-QUARTERS OF THE WAY THROUGH YEAR TWO OF OUR STRATEGIC PLAN, WHICH THIS BOARD APPROVED IN DECEMBER 2015. AND THERE ARE -- WE FINALLY CALL THESE THE BIG SIX, WHICH ARE THE SIX BOLD GOALS WE PUT FORWARD. THEY'RE THE SIX BOLD GOALS THAT WE ARE TARGETING, BUT ALSO MEASURING, AND WE HAVE METRICS ATTACHED TO ALL OF THESE.

AND SUFFICE IT TO SAY THAT WE'RE ON TRACK, ON TARGET TO MEET THESE GOALS AS BOLD AS THEY ARE. AND IN PARTICULAR, THEY JUST RELATE TO ACCELERATING THE DEVELOPMENT OF TREATMENTS TO PATIENTS EITHER THROUGH CREATION AND LAUNCHING OF CRITICAL INFRASTRUCTURE, ENACTING AND REFINING REGULATORY PATHWAYS IN ORDER TO FACILITATE THE SAFE TESTING AND ADVANCEMENTS OF THESE TREATMENTS TO PATIENTS, AS WELL AS SUPPORTING, IDENTIFYING, AND HELPING SHAPE PROMISING TECHNOLOGIES THAT WOULD BE APPLIED TO A VARIETY OF DEBILITATING AND FATAL DISEASES AND CONDITIONS, AND CONTINUED PARTNERSHIP WITH INDUSTRY BECAUSE INDUSTRY DOES NEED TO BE ACTIVELY INVOLVED IN TAKING THIS TO COMMERCIALIZATION AND MAKING IT WIDELY AVAILABLE TO ALL PATIENTS.

IN LAUNCHING THE STRATEGIC PLAN, WE REVAMPED MANY THINGS, HOW WE DID BUSINESS, MADE THINGS MORE PREDICTABLE, OUR FUNDING PROCESS.

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EVERYTHING THAT WE DID AT THE AGENCY WAS DELIBERATE, MEASURED, AND PREDICTABLE AND TRANSPARENT. AND BECAUSE OF THIS, SOME OF YOU HAVE SEEN THIS BEFORE, WE HAVE CONTINUED TO SEE THAT THE OPERATIONAL EXCELLENCE AND EFFICIENCIES IN TERMS OF NUMBERS OF REVIEWS THAT ARE CONDUCTED PER YEAR, DOING IT IN A COST-EFFECTIVE FASHION, AND DECREASING THE TIME TO CONTRACTING, AND INCREASING THE NUMBER THAT'S COMING INTO OUR PORTFOLIO. THESE HAVE ALL SHOWED TRENDS IN A POSITIVE WAY, AND THIS HAS ENABLED US TO MARKEDLY GROW THE PORTFOLIO.

JUST AS AN EXAMPLE OF THIS, FOR OUR CLINICAL PROGRAMS, FOR EXAMPLE, WHEN ONE LOOKS AT THE ACTIVITY IN TERMS OF CLINICAL APPLICATIONS COMING IN, THE TREND INCREASED OVER EVEN THE PAST THREE YEARS, AND THE 2017 NUMBER IS JUST AN ANNUALIZED NUMBER. WE EXPECT IT MAY BE EVEN LARGER THAN THAT.

ON THE RIGHT THE SLIDE JUST SPEAKS TO THE QUALITY OF THE PROGRAMS COMING IN. JUST TO REMIND EVERYBODY, AND YOU WILL HEAR ABOUT THIS AGAIN LATER WHEN DR. SAMBRANO PRESENTS THE CLINICAL PROGRAM RECOMMENDATIONS, A TIER I FUNDING REPRESENTS PROJECTS THAT OUR GRANTS WORKING GROUP FIND MERITORIOUS AND RECOMMEND TO OUR BOARD FOR FUNDING,

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AND THE PERCENT OF APPLICATIONS RECEIVING THIS TIER I SCORE HAS CONTINUED TO INCREASE. THIS IS NOT BY ACCIDENT. THERE HAS BEEN ACTIVE ENGAGEMENT AND INVOLVEMENT BY THE CIRM TEAM IN ORDER TO SHAPE THESE PROGRAMS EVEN IN THE PLANNING STAGES PRIOR TO COMING IN AND THEN DURING IMPLEMENTATION WITH OUR CLINICAL ADVISORY PANEL. IT'S IMPROVED THE PROGRESS OF THESE PROJECTS AND MEETING THEIR MILESTONES. AND THERE'S CONTINUED INCREASE AND CONSISTENT INDUSTRY INVOLVEMENT. SO WE'RE PLEASED FOR THAT.

SO WITH ACTIVE CIRM ENGAGEMENT, THERE'S BEEN AN INCREASE IN PROGRESSION EVENTS OVER THE PAST THREE YEARS, MEANING PROJECTS GOING FROM THE EARLY STAGE RESEARCH TO GOING TO THE TRANSLATIONAL STAGE TO GOING TO CLINICAL TESTING. WE'VE COUNTED ABOUT 37 PROGRESSION EVENTS, WHICH IS MARKEDLY INCREASED FROM 27 THAT WE COULD THINK OF WAY BEFORE THE STRATEGIC PLAN AND THE NEW SYSTEMS WERE PUT IN PLACE. REDUCED THE TIME TO IND. WE NOW HAVE SEVERAL PROGRAMS THAT HAVE OBTAINED AN IND WITH A CLINICAL 1 AWARD WITHIN 18 MONTHS. WE ASKED FOR THAT AND THEY DELIVERED ON IT. AND THIS HAS RESULTED IN THE GROWTH AND NUMBER OF HIGH QUALITY CLINICAL PROGRAMS.

SO OUR GOAL IN THE STRATEGIC PLAN, AS YOU

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RECALL, IS 50 NEW TRIALS BY 2020. PRIOR TO LAUNCHING THE STRATEGIC PLAN, CIRM IN A LITTLE BIT OVER TEN YEARS HAD AMASSED 17 CLINICAL TRIALS. IN JUST LESS THAN TWO YEARS OF LAUNCHING THE STRATEGIC PLAN, WE DOUBLED THE PORTFOLIO. AFTER TODAY'S MEETING, IT WILL EXCEED THAT AND BY THE END OF THE YEAR AS WELL. SO THE BOLD GOAL IS NEARLY QUADRUPLING OUR PORTFOLIO IN THE SPAN OF FIVE YEARS.

AND I WON'T GO INTO THIS BECAUSE DR. TALIB WILL PRESENT OUR CLINICAL PORTFOLIO WITH A FOCUS ON A PARTICULAR AREA AS WE DO EVERY QUARTER. AND WHAT THIS TRANSLATES INTO IS CONTINUED CLINICAL TRIAL ACTIVITY AS WE CAN TRACK WITH PATIENT ENROLLMENT, SUBJECT ENROLLMENT, INTO THE TRIALS. EVERY SINGLE POINT THAT GOES INTO THIS REPRESENTS A COUPLE OF THINGS. ONE IS ACTIVE PARTNERSHIP WITH THE PATIENTS WHO ARE INVOLVED IN THESE EARLY STAGE TRIALS AS WELL AS A LOT OF WORK THAT WENT INTO GETTING THESE PROGRAMS TO THE STAGE WHERE THEY CAN BE TESTED IN THE CLINICS.

I'LL JUST END BY REMINDING EVERYBODY OF OUR BIG BUCKET. WE CALL IT THE BIG BUCKET, WE CALL THE RESEARCH FUND AT CIRM, THE AMOUNT OF MONEY THAT'S AVAILABLE TO FUND THE GRANTS. CALL IT BIG BUCKET VERSUS LITTLE BUCKET, WHICH IS ADMINISTRATIVE

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FUND. WE HAVE APPROXIMATELY 414 MILLION REMAINING UNCOMMITTED AS OF TODAY. AND THE TOTAL COMMITTED RESEARCH DOLLARS IS $2.34 BILLION SINCE THE INITIATIVE WAS LAUNCHED.

SO THANK YOU VERY MUCH FOR YOUR ATTENTION.

CHAIRMAN THOMAS: THANK YOU, DR. MILLAN.

(APPLAUSE.)

CHAIRMAN THOMAS: OKAY. MOVE ON TO THE NEXT TOPIC, CONSIDERATION OF APPLICATIONS SUBMITTED IN RESPONSE TO THE INFRASTRUCTURE 4 OR ALPHA CLINIC NETWORK EXPANSION AWARDS. AT THIS POINT I'D LIKE TO TURN THE MICROPHONE OVER TO SUPERVISOR SHEEHY.

SUPERVISOR SHEEHY: THANK YOU, CHAIRMAN THOMAS. AND SO NOW I BELIEVE WE'RE SITTING AS THE APPLICATION REVIEW SUBCOMMITTEE.

AND, DR. SAMBRANO, I BELIEVE YOU HAVE SOME INTRODUCTORY SLIDES, AND THEN I THINK -- WHAT IS THE PROCESS? YOU'LL DO SOME SLIDES, AND THEN WE'LL HEAR FROM THE APPLICANTS?

DR. SAMBRANO: SO WHAT WE WILL DO IS I'LL GO THROUGH AN OVERVIEW OF THE PROGRAM, THE REVIEW PROCESS. I'LL INTRODUCE EACH OF THE APPLICANTS. THEY WERE INSTRUCTED THEY HAVE FIVE MINUTES IN TERMS OF A PRESENTATION. IF YOU HAVE QUESTIONS FOR THEM, YOU CAN CERTAINLY ENGAGE THEM. WE WILL GO THROUGH

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ALL THREE, AND THEN WE TURN IT BACK TO YOU.

SUPERVISOR SHEEHY: SO AFTER THEY PRESENT, WE'LL TAKE QUESTIONS FROM THEM?

DR. SAMBRANO: YES.

SUPERVISOR SHEEHY: WE'LL ASK QUESTIONS OF THEM.

MR. TOCHER: JEFF, IF I COULD JUST REMIND THE FOLKS ON THE BOARD THAT FOR THOSE MEMBERS WHO HAVE A CONFLICT WITH ANY OF THE INSTITUTIONS THAT ARE HERE TODAY FOR FUNDING, YOU WILL NOT BE ABLE TO PARTICIPATE IN THIS DISCUSSION UNTIL THE APPLICATION THAT YOU HAVE CONFLICT WITH IS DISPOSED OF.

SUPERVISOR SHEEHY: SO REALLY -- LET'S JUST BE CLEAR. IF YOU HAVE A CONFLICT WITH ANY OF THE APPLICANTS, YOU CANNOT HAVE A CONVERSATION WITH ANY OF THE APPLICANTS.

MR. TOCHER: THAT'S CORRECT.

SUPERVISOR SHEEHY: SO IT'S BLANKET. SINCE WE WON'T BE TAKING VOTES ON THEM UNTIL AFTER WE'VE HEARD THEM AND HAD A DISCUSSION, THEN IT REALLY IS ONLY IN THE CONSIDERATION OF THE APPLICATIONS AFTER WE --

MR. TOCHER: HAVE DISPOSED OF --

SUPERVISOR SHEEHY: THE ONE WITH WHICH SOMEONE HAS A CONFLICT.

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MR. TOCHER: THAT'S CORRECT.

SUPERVISOR SHEEHY: GREAT. THANK YOU.

DR. SAMBRANO: GOOD MORNING, EVERYONE. THANK YOU VERY MUCH. I'M GOING TO PRESENT AN OVERVIEW OF THE ALPHA STEM CELL CLINICS PROGRAM.

SO ONE OF THE CHALLENGES THAT WE HAVE AT CIRM IN ALIGNING IT WITH OUR MISSION IS TO ACCELERATE STEM CELL TREATMENTS. AND IT IS A CHALLENGE IN THAT IND-ENABLING STUDIES TO GET TO THE CLINIC ARE DISPROPORTIONATELY MUCH LONGER FOR CELL THERAPY THAN IT IS FOR NONCELLULAR TREATMENTS. SO ONE OF THE THINGS THAT WE TRY TO DO IS BUILD INFRASTRUCTURE PROGRAMS THAT CAN HELP PUSH AND/OR PULL IN ANY WAY ANY OF THESE PROJECTS THROUGH THAT STAGE AND ALLOW THEM TO GET TO THE CLINIC FASTER.

AS YOU KNOW, WE HAVE SEVERAL PROGRAMS ALREADY IN PLACE. SO THERE WAS THE TRANSLATING CENTER, AN ACCELERATING CENTER THAT YOU'VE CONSIDERED BEFORE. THOSE HAVE ACTUALLY NOW MERGED INTO WHAT IS NOW CALLED THE STEM CELL CENTER. SO THEIR RESPONSIBILITY IS INCLUDING REGULATORY SUBMISSIONS, TRIAL MANAGEMENT, PROCESS DEVELOPMENT, DOING TOX STUDIES AND SO ON FOR AND WITH APPLICANTS OR GRANTEES.

WE ALSO HAVE ESTABLISHED THE ALPHA CLINICS

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NETWORK, WHICH I WILL TALK A LITTLE BIT MORE ABOUT. THE ALPHA STEM CELL CLINICS WAS LAUNCHED IN DECEMBER OF 2014. AND THREE SITES FROM THAT INITIAL PROGRAM BECAME ACTIVE IN 2015. SO THE THREE SITES THAT WERE ESTABLISHED WERE AT THE CITY OF HOPE, UCSD, AND A CONSORTIUM BETWEEN UCLA AND UCI.

THE FUNCTIONS OF ANY OF THESE ALPHA CLINICS, THEY HAVE CERTAIN CORE FUNCTIONS THAT THEY HAVE. ALL OF THESE ARE PROPORTIONAL TO THE NEEDS OF THE CLINICAL TRIAL THAT COMES TO THEM; BUT, IN GENERAL, THEY ARE RESPONSIBLE FOR CONTRACTING AND NEGOTIATING CLINICAL TRIAL AGREEMENTS TO THEIR SITE, ASSISTING WITH IRB SUBMISSIONS AND APPROVAL, PATIENT RECRUITMENT SCREENING, ENROLLMENT, CLINICAL TRIAL MONITORING, PATIENT COORDINATION SCHEDULING, AND DATA MANAGEMENT.

SO OUR GOAL FOR THIS NEW PROGRAM WAS TO EXPAND THAT NETWORK. WE FEEL IT HAS BEEN SUCCESSFUL THUS FAR AND WANTED TO ADD UP TO TWO ADDITIONAL SITES TO THIS NETWORK. IN ADDITION TO THAT, WE WANTED TO INCLUDE A NEW PHYSICIAN TRAINING COMPONENT. THAT'S A PART OF THOSE NEW ALPHA CLINIC SITES. AND THEN ALSO TO BRING ANYTHING THAT WOULD ENHANCE THE VALUE OF THE NETWORK OVERALL. SO THIS COULD BE GREATER GEOGRAPHIC REACH BY BRINGING ALPHA

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CLINICS INTO THE REGION WHERE THEY'RE OTHERWISE NOT AVAILABLE, OR TO BRING IN NEW AREAS OF EXPERTISE. WE WANTED THEM ALL TO CREATE A SUSTAINABLE PROGRAM SO THAT, ONCE CIRM HAS COMPLETED THEIR FUNDING OF THESE PROGRAMS, THAT THEY ARE ABLE TO EXIST AND MOVE FORWARD WITH THESE CORE ACTIVITIES. AND WE WOULD PROVIDE UP TO EIGHT MILLION FOR FOUR YEARS FOR EACH OF THE GRANTEES.

LITTLE BIT ABOUT THE REVIEW PROCESS. SO AS WE HAVE DONE WITH OTHER INFRASTRUCTURE PROGRAMS, THE REVIEW OF THESE IS A LITTLE BIT DIFFERENT. WE HAVE FOUR BASIC -- IT WASN'T REALLY THAT OMINOUS WHEN WE DID THE REVIEW. WE HAD FOUR MAJOR COMPONENTS TO THE REVIEW. THE FIRST WAS THE GRANTS WORKING GROUP PANEL HAS A DISCUSSION INITIALLY WHEN THEY COME TOGETHER ABOUT THE APPLICATION FOR ABOUT 20 TO 25 MINUTES. AND ONE OF THE UNIQUE FEATURES IS THAT WE BRING THE APPLICANT TO THE PANEL SO THAT THEY CAN HAVE A FACE TO FACE. SO THE APPLICANTS DO A PITCH, A PRESENTATION, ABOUT THE VALUE OF THEIR PROPOSED ALPHA CLINICS. WE GIVE THEM ABOUT FIVE MINUTES, AND THEN THERE'S A DIRECT Q AND A OF THE APPLICANT WITH THE GWG. SO THE GWG ASKS CLARIFYING QUESTIONS OR WHATEVER THEY NEED IN ORDER TO PROPERLY ASSESS THESE APPLICATIONS. THEN THEY HAVE A WRAP-UP

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DISCUSSION AND THEY SCORE. SO WE DID THIS WITH ALL OF THE APPLICATIONS THAT CAME THROUGH THE PROGRAM.

THE CRITERIA OR KEY QUESTIONS THAT THEY USED IN ORDER TO ASSESS THESE PROPOSALS WERE THESE THREE BROAD QUESTIONS. WILL THE PROPOSED ALPHA CLINIC ACCELERATE THE COMPLETION OF STEM CELL CLINICAL TRIALS AND ENHANCE THE VALUE OF THE NETWORK SUCH THAT IT IS ONE THAT IS SUSTAINABLE? HAS THE APPLICANT DEVELOPED AN APPROPRIATE PLAN DESIGNED TO SUCCESSFULLY ESTABLISH AND OPERATIONALIZE THE ALPHA CLINIC? AND IS THE PROPOSAL OVERALL FEASIBLE? CAN THEY DO NOT ONLY THE CORE ACTIVITIES THAT WE ARE REQUIRING OF THEM, BUT ALSO THOSE THAT THEY ARE BRINGING FORWARD TO ENHANCE THE VALUE OF THAT ALPHA CLINIC?

THE SCORING SYSTEM THAT WAS USED IN THIS REVIEW WAS A SCORE -- THE SYSTEM OF 1, 2, OR 3, WHERE A SCORE OF 1 MEANS IT HAS EXCEPTIONAL MERIT AND, FROM THE PERSPECTIVE OF THE GWG, THAT THIS IS SOMETHING THAT THEY WOULD RECOMMEND FOR FUNDING. A SCORE OF 2 MEANS IT NEEDS IMPROVEMENT, COULD BE RESUBMITTED. THERE WERE NONE THAT RECEIVED THE SCORE, SO IT DOESN'T APPLY. AND A SCORE OF 3, THAT IT'S SUFFICIENTLY FLAWED THAT IT DOES NOT WARRANT FUNDING.

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ALL RIGHT. THE GUIDANCE THAT WE GAVE TO GWG REVIEWERS AS THEY ENGAGED IN THIS REVIEW WAS THAT WE WERE LOOKING IDEALLY TO EXPAND THE PROGRAM BY TWO SITES. WE TOLD THEM THAT THEY'RE NOT REQUIRED TO SELECT EITHER A SINGLE OR TWO WINNING APPLICATIONS. IF THEY FELT THAT NONE OF THE APPLICATIONS THAT WERE CONSIDERED WERE DESERVING OF A TIER I SCORE, THEY DIDN'T HAVE TO SCORE IT IN THAT TIER. AND THEN BASED ON THEIR RECOMMENDATIONS, THE GOVERNING BOARD WILL DETERMINE WHICH APPLICANTS RECEIVE AN AWARD.

AND THEN A REMINDER, JUST ESPECIALLY IF YOU HAVEN'T BEEN TO A BOARD MEETING BEFORE. USUALLY AT THE END OF EACH REVIEW, REGARDLESS OF THE REVIEW TYPE, WE HAVE A VOTE BY THE PANEL THAT INCLUDES ALL MEMBERS, THE SCIENTIFIC MEMBERS, PATIENT ADVOCATE MEMBERS, AND THEIR ASSESSMENT OF WHETHER THE REVIEW PROCESS AND THE REVIEW ITSELF WAS CONDUCTED IN AN APPROPRIATE MANNER. SO IN THIS REVIEW, THE VOTE WAS UNANIMOUS IN FAVOR OF BOTH STATEMENTS BY THE PANEL.

SO AT THIS TIME I'M GOING TO JUST INTRODUCE THE FIRST APPLICANT THAT WILL PRESENT. THE TITLE OF THEIR APPLICATION IS "THE ALPHA CLINIC, A PARTNER IN THE ADVANCEMENT OF CELL THERAPY RESEARCH." THIS APPLICATION RECEIVED A 1, AND THERE

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WERE SEVEN MEMBERS WHO SCORED IT IN THE TOP TIER. THERE WERE FOUR MEMBERS WHO GAVE IT A SCORE OF 2 AND TWO MEMBERS THAT GAVE IT A SCORE OF 3.

SO DR. TIM HENRY IS GOING TO BE PRESENTING FOR THIS APPLICATION.

DR. HENRY: THANK YOU. AND CONGRATULATIONS, DR. MILLAN. GOOD MORNING. I'M TIM HENRY. I'M THE CHIEF OF CARDIOLOGY AT CEDARS AND THE PROPOSED DIRECTOR FOR THE CEDARS ALPHA CLINIC. WE ARE EXTREMELY EXCITED TO BE PART OF THE CIRM ALPHA CLINIC NETWORK. I STRONGLY BELIEVE IN THIS CONCEPT AND HAVE BEEN ACTIVELY INVOLVED IN OUR SUCCESSFUL REGENERATIVE MEDICINE CLINIC FOR MORE THAN TEN YEARS. SO I KNOW PERSONALLY HOW THIS CAN WORK.

THIS IS OUR PROPOSED NETWORK AS WE PRINT. WHAT I WANT TO DO IS WE APPRECIATED THE COMMITTEE RECOGNIZED THE STRENGTHS OF OUR ALPHA CLINIC APPLICATION, WHICH I'D LIKE TO SUMMARIZE. AN ACADEMIC MEDICAL CENTER WITH A VERY LARGE CLINICAL VOLUME; AN ESTABLISHED AND SUCCESSFUL REGENERATIVE MEDICINE CLINIC ALREADY THAT COLLABORATES ALL ACROSS, NOT ONLY THE STATE, BUT THE NATION; AN EXTENSIVE PROVEN EXPERIENCE IN OVER 40 CARDIOVASCULAR CELL THERAPY TRIALS, WHICH WE THINK

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IS A VERY IMPORTANT DISEASE PROCESS, WITH OVER 500 PATIENTS ENROLLED, 11 DIFFERENT CELL TYPES, AND SEVEN DIFFERENT METHODS OF DELIVERY; EXTENSIVE NEUROSCIENCE EXPERIENCE IN ALS, DUCHENNE'S MUSCULAR DYSTROPHY, AND STROKE, WHICH WE ALSO BELIEVE EXPANDS THE DISEASE TARGET FOR THE NETWORK.

IT'S IMPORTANT TO KNOW TO BRING YOU UP TO DATE THAT OUR TWO LEAD CLINICAL TRIALS, BOTH CIRM SPONSORED, ONE IN ALS AND ONE IN PULMONARY HYPERTENSION, ARE BOTH ACTIVELY ENROLLING, AND THEY'RE PART OF A ROBUST PIPELINE OF 20 ONGOING AND PLANNED CLINICAL TRIALS.

WE HAVE A PROVEN TRACK RECORD IN CELL THERAPY TRIAL LEADERSHIP, TRIAL DESIGN, STEERING COMMITTEE, NATIONAL PI AND DSMB, WHICH SHOWS OUR COLLABORATIVE NATURE OF WORKING WITH OTHER INSTITUTIONS. WE HAVE AN ESTABLISHED TRAINING PROGRAM FOR CELL THERAPY RESEARCH. IN FACT, WE HAVE AN NIH TRAINING GRANT THAT WOULD BE AVAILABLE TO THE CIRM NETWORK TO TRAIN RESEARCH NURSES AS WELL AS CARDIOLOGY FELLOWS.

WE HAVE AN ESTABLISHED EXPERIENCE IN SATELLITE NETWORK MANAGEMENT AND COLLABORATION AS PART OF THE NIH CELL THERAPY NETWORK AND MULTIPLE CENTER CLINICAL TRIALS.

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WE ALSO APPRECIATE THE OPPORTUNITY TO REVIEW THE REVIEWER RECOMMENDATIONS AND TO ADDRESS WHICH WE FELT WERE MINOR CONCERNS. THE ONE IS TO ADD ONE TO TWO FELLOWS BEING TRAINED. WE'D BE ABSOLUTELY DELIGHTED TO DO THIS, CAN EASILY ACCOMPLISH THIS. AND, IN FACT, OUR GOAL WOULD BE THEN TO HAVE TWO TO FOUR APPLICANTS PER YEAR, TWO IN CARDIOLOGY, ONE IN NEURO, AND ONE IN ONCOLOGY, ASSUMING THAT WE HAD WELL-QUALIFIED APPLICANTS, BUT WE HAVE MORE THAN ENOUGH BANDWIDTH TO ACCOMPLISH THIS. AND THEY'RE ALREADY DOING EXTENSIVE TRAINING AT CEDARS, AS YOU KNOW.

THE SECOND QUESTION WAS HOW WOULD THE CEDARS ALPHA CLINIC MANAGE OR HANDLE COMPETING INTERESTS BETWEEN RESEARCHERS AT CEDARS AND THEIR COMPETITION? SO WE HAVE SUCH A LARGE PORTFOLIO NOW. THIS IS AN ONGOING ISSUE ALL THE TIME. AS PART OF OUR STRUCTURE, ANY COMPETING INTEREST, IF THERE EVER WAS A CONFLICT, AND TO THE TIME I'VE BEEN AT CEDARS, THERE NEVER HAS BEEN A CONFLICT, WE WOULD ACTUALLY BE ADJUDICATED BY A COMMITTEE OF THREE UNINVOLVED DEPARTMENT CHAIRS THAT ARE APPOINTED BY THE DEAN. SO WE WOULD HAVE A STRUCTURE IN PLACE FOR THAT.

AND THEN THE LAST QUESTION WAS THE APPLICANT'S SUSTAINABILITY PLAN SHOULD INCLUDE

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SUPPORT FOR STEM CELL CLINICAL TRIALS OF POTENTIAL COMPETITORS TO ITS INTERNAL PIPELINE. WE FELT THIS IS VERY IMPORTANT TO EMPHASIZE THIS. WHILE CERTAINLY WE'VE HAD A GOOD, STRONG INTERNAL PIPELINE, WHICH WE ARE PROUD OF, I'D LIKE TO POINT OUT THAT LESS THAN 20 PERCENT OF OUR ENROLLMENT IN CLINICAL TRIALS HAS BEEN IN THE INTERNAL PIPELINE. IN FACT, I'VE BEEN NATIONAL PI FOR AT LEAST SEVEN TRIALS THAT ARE NOT CONNECTED TO CEDARS AT ALL.

SO WE BELIEVE THIS IS A STRENGTH OF OURS, THAT WE ACTUALLY HAVE WIDE PARTICIPATION ACROSS MANY THINGS, AS DO OUR NEUROSCIENCE.

SO WITH THAT, I WILL SUMMARIZE AND SAY AGAIN WE WOULD BE ABSOLUTELY DELIGHTED TO BE PART OF THE ALPHA CLINIC NETWORK. WE THINK WE BRING A LOT TO THE TABLE, AND CERTAINLY BEING PART OF THE NETWORK WOULD HELP US ACCOMPLISH OUR MISSION. SO THANK YOU VERY MUCH.

SUPERVISOR SHEEHY: SO I HAD SOME QUESTIONS, BUT I ALSO -- I THINK OTHER BOARD MEMBERS MAY HAVE QUESTIONS. WE DID NOT GET -- THIS IS 10103. WHAT WAS THE VOTE AT THE -- WE DIDN'T HAVE A DISCUSSION OF WHAT THE VOTE WAS AT THE -- THERE IT IS. THAT'S WHAT I WAS ASKING TO BE SHOWN SO WE COULD ALL GET THE BENEFIT OF THAT.

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WELL, I HATE TO JUST JUMP IN IF OTHER PEOPLE HAVE QUESTIONS. STEVE, I'M SURE YOU HAVE QUESTIONS. DOES ANYBODY HAVE QUESTIONS? MAYBE STEVE AND THEN CHAIRMAN THOMAS.

DR. JUELSGAARD: SO I'M GOING TO FOCUS ON TWO COMMENTS THAT WERE IN THE PRESENTATION THAT WAS PROVIDED TO US PRIOR TO THIS MEETING. THE FIRST, AND YOU'VE PROBABLY SEEN THESE, THE FIRST IS THE PROPOSED CLINIC WILL EXPAND EXPERTISE -- THE PROPOSED CLINIC HAS THE POTENTIAL TO EXPAND GEOGRAPHIC REACH INTO NORTHERN CALIFORNIA BY ENGAGING IN SATELLITE SITES; HOWEVER, REVIEWERS QUESTIONED THE FEASIBILITY OF CONDUCTING STEM CELL TRIALS AT PROPOSED SATELLITE SITES.

BY WAY OF BACKGROUND, IT'S MY UNDERSTANDING, AT LEAST, THAT IN THIS REQUEST FOR PROPOSALS FOR ADDITIONAL ALPHA CLINIC STEM CELL CLINICS, WE WERE REALLY LOOKING AT GEOGRAPHICALLY THE NORTHERN PART OF THE STATE AS OPPOSED TO THE SOUTHERN PART OF THE STATE, WHICH IS ALREADY WELL SERVED BY THE THREE EXISTING ONES. SO THE QUESTION IS HOW DO YOU INTEND TO CONDUCT STEM CELL TRIALS IN NORTHERN CALIFORNIA TO KIND OF BOIL IT DOWN?

DR. HENRY: YES. SO ONE OF THE THINGS, AND WE HAD IN THE APPLICATION, SO I'VE HAD EXTENSIVE

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EXPERIENCE OVER THE LAST TEN YEARS DEVELOPING SATELLITE NETWORKS, INCLUDING AS PART OF THE NIH CELL THERAPY NETWORK. AND WE HAVE TWO PLANS. THE INITIAL PLANS WERE TO BE TWO. NO. 1, OUR CARDIOLOGY FELLOWSHIP ALREADY SHARES FELLOWSHIP WITH KAISER. AND SO WE HAVE THREE SITES IN NORTHERN CALIFORNIA KAISER WITH FORMER FELLOWS WHO ARE ACTUALLY NOT ONLY WILLING, BUT EXCITED TO BE PART OF A NETWORK AT NORTHERN CALIFORNIA KAISER THAT WOULD ALSO INCLUDE KAISER SOUTHERN CALIFORNIA AND THROUGHOUT THE STATE. AND THEN THE SECOND ORGANIZATION IS SUTTER HEALTH AND CALPACIFIC, AND IN PARTICULAR DICK SHAW, WHO'S EXCITED TO BE PART OF IT. SO ALL OF THOSE ARE ALREADY THERE, AND THIS WOULD START FROM A CARDIOVASCULAR STANDPOINT AND WOULD HAVE IN PLACE TO BE BOTH WITH HEART FAILURE, WITH REFRACTURING ANGINA, AND WITH PERIPHERAL ARTERIAL DISEASE AND WOULD BE READY TO PARTICIPATE LIKE ALMOST IMMEDIATELY.

DR. JUELSGAARD: THANK YOU. SO THE SECOND QUESTION, AND YOU SORT OF ADDRESSED THIS IN YOUR PRESENTATION, BUT I'M GOING TO COME BACK AROUND TO IT BECAUSE I WANT TO UNDERSTAND HOW, AND YOU WON'T HAVE THE ANSWER TO QUESTION, BUT IT'S AROUND COMPETING CLINICAL TRIALS AND STEM CELL THERAPY. SO

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THE COMMENT WAS FROM THE REVIEWERS, "IT WAS UNCLEAR WHETHER THE APPLICANT WOULD SUPPORT CLINICAL TRIALS FOR POTENTIAL COMPETITORS OF ITS OWN STEM CELL PLATFORM TECHNOLOGY." SO WE HAVE THREE EXISTING STEM CELL CENTERS OR ALPHA CLINIC CENTERS. WE HAVE TWO OTHERS THAT WILL BE COMING TO THE DAIS HERE BEFORE LONG. AND I'VE NEVER HEARD OF THIS ISSUE BEFORE, AND IT MAY EXIST, I'M JUST NOT AWARE OF IT, OF COMPETING STEM CELL PLATFORMS AND HAVING TO WORRY ABOUT THEM.

SO YOU JUST INDICATED YOU WILL HAVE A SYSTEM WHERE THREE PEOPLE WILL REVIEW AND MAKE A DECISION. BUT THE FACT OF THE MATTER IS THAT THAT ACTUALLY HAS TO OCCUR, THAT THAT BECOMES A HURDLE TO GET OVER. SO WHY ISN'T IT THAT YOU'RE JUST ABLE TO WAIVE THAT ALTOGETHER AND BE ABLE TO TAKE ALL COMERS?

DR. HENRY: I DIDN'T QUITE UNDERSTAND WHERE THIS CAME FROM BECAUSE IF YOU LOOK AT THE PATIENTS THAT WE'VE ENROLLED AND THE EXTENT OF WHAT WE'VE ENROLLED IN TRIALS, WE'VE ENROLLED IN TRIALS FOR MULTIPLE DIFFERENT CELLS, DIFFERENT DELIVERY. AND I'M ASSUMING THAT THEY'RE INTERNAL FROM THE CAPRICOR, WHICH HAVE BEEN CIRM SPONSORED, BUT THERE'S BEEN NO PREFERENCE SHOWN FOR THAT. IN FACT,

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I HAVE BEEN NATIONAL PI OF THREE OTHER TRIALS IN THE LAST YEAR THAT HAVE BEEN PUBLISHED THAT HAVE NOTHING TO DO WITH ANY INTERNAL CANDIDATES.

SO WE WORK EVERY DAY WITH REALLY A VERY WIDE SPECTRUM OF COMPANIES. WE HAVE MORE THAN 20 TRIALS THAT WE'RE WORKING ON. SO I WASN'T REALLY SURE, AND IT CERTAINLY HAS NEVER BEEN A PROBLEM, AND IT WON'T BE A PROBLEM. AND I PERSONALLY HAVE NO CONNECTION, BY THE WAY, WITH THE INTERNAL DEVELOPMENT CANDIDATES.

DR. JUELSGAARD: THANK YOU.

SUPERVISOR SHEEHY: CHAIRMAN THOMAS.

CHAIRMAN THOMAS: WHEN THE ALPHA CLINIC NETWORK WAS FIRST ESTABLISHED, IT REALLY IS A ONE-OF-A-KIND NETWORK ANYWHERE. AND THE PURPOSE OF IT WAS TO ATTRACT BEST-IN-CLASS CLINICAL TRIALS, NOT JUST THAT WERE CIRM-FUNDED, BUT THAT ARE FUNDED ELSEWHERE AND DID NOT NEED TO BE ORIGINATING IN CALIFORNIA. THEY COULD HAVE A CLINICAL TRIAL COMPONENT OUTSIDE THE STATE, ETC. SO WE ACTIVELY ENCOURAGED RECRUITMENT OF TRIALS TO PUT IN THERE.

SO MY QUESTION TO YOU IS HOW DO YOU GUYS CURRENTLY RECRUIT CLINICAL TRIALS TO BE DONE AT CEDARS? AND HOW WOULD GETTING THE ALPHA CLINIC DESIGNATION AFFECT THAT RECRUITMENT EFFORT IF AT

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ALL?

DR. HENRY: SO WE HAVE SUCH AN ACTIVE PROGRAM ALREADY THAT THE ACTUAL SPONSORS, AND NIH, ETC., FREQUENTLY APPROACH US. AND WE HAVE SUCH A LARGE CLINICAL VOLUME SO THAT CARDIOVASCULAR, FOR EXAMPLE, WE HAVE MULTIPLE HEART FAILURE TRIALS, REFRACTURING ANGINA TRIALS, PERIPHERAL ARTERIAL DISEASE, STROKE, ALL ALREADY MULTIPLE TRIALS IN THOSE SETS. AND SO USUALLY THEY APPROACH US. AND, IN FACT, I'VE BEEN INVOLVED WITH THE DESIGN OF MOST OF THESE TRIALS THAT ARE MULTICENTER AND BOTH NATIONAL AND INTERNATIONAL.

BUT THIS WILL ONLY ENHANCE IT, AND THE NEUROSCIENCE HAS GROWN TREMENDOUSLY. SO SINCE WE ACTUALLY PUT THE APPLICATION IN, THEY'VE ACTUALLY NOT ONLY GOTTEN THEIR OWN LEAD TRIAL UNDER WAY WITH ALREADY FIVE TREATED PATIENTS, SO ENROLLING AHEAD OF SCHEDULE, BUT THEY ACTUALLY WERE JUST ASKED TO BE PART OF A MULTICENTER TRIAL WHERE ONLY FIVE SITES WERE CHOSEN. SO IT'S A COMBINATION OF WE KNOW WHAT TRIALS ARE GOING ON.

SO LAST WEEK, FOR EXAMPLE, I WAS IN HOUSTON AT A NATIONAL STEM CELL MEETING WHERE REALLY I'M AWARE OF ALL TRIALS THAT ARE GOING ON IN CARDIOVASCULAR AS WELL AS OTHER AREAS. SO AN ACTIVE

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RECRUITING BOTH WAYS.

BEING PART OF THE ALPHA CLINIC NETWORK, I THINK, ALLOWS US TO NOT ONLY RECRUIT MORE TRIALS, BUT TO SHARE THOSE WITH ALL THE OTHER SITES IN THE TRIAL AND BE PART OF IT. SO I THINK IT ENHANCES IT. I THINK GOING FROM THREE TO FIVE CENTERS IS A TREMENDOUS IDEA, AND WE'LL REALLY BE ABLE TO INCREASE OUR PORTFOLIO DRAMATICALLY.

CHAIRMAN THOMAS: THANK YOU.

SUPERVISOR SHEEHY: OS.

DR. STEWARD: SO GOING BACK TO THE GEOGRAPHIC REACH QUESTION, THIS WAS SOMETHING OF A TOPIC OF DISCUSSION, AS YOU KNOW. CAN YOU TALK A LITTLE BIT MORE ABOUT HOW YOUR PROGRAM WOULD REALLY GO BEYOND THE AREAS THAT ARE CURRENTLY SERVED? WHEN WE TALK ABOUT SOUTHERN AND NORTHERN, WE OFTEN THINK ABOUT THE GREATER LOS ANGELES, SAN DIEGO, AND ALL OF THAT, AND THEN NORTHERN IS LIKE 50, 60 MILES FROM HERE, BUT THERE'S A WHOLE BIG SWATH OF CALIFORNIA THAT GOES BEYOND THIS 60-MILE WIDE STRIP THAT RUNS ALONG THE COAST.

WHERE ARE YOU THINKING ABOUT DEVELOPING THESE OTHER SITES? WHAT PARTS OF CALIFORNIA WOULD BE SERVED?

AND THEN SECOND, COULD YOU JUST UNPACK A

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LITTLE BIT HOW YOU SEE THESE SITES REALLY BEING DEVELOPED AT A DISTANCE FROM THE MOTHER SHIP, SO TO SPEAK?

DR. HENRY: THERE'S TWO PARTS TO THAT QUESTION. FIRST OF ALL, FROM CEDARS WE ALREADY HAVE AN EXTREMELY LARGE GEOGRAPHIC FOOTPRINT. SO WE GET A LOT OF PATIENTS FROM PALM SPRINGS, SORT OF EAST OF US REGION, ALL THE WAY OUT TO PHOENIX AND TO LAS VEGAS. A LOT OF PATIENTS ALREADY COME TO US FROM SAN DIEGO AND THAT SOUTHERN REGION AND ALL THE WAY UP TO BAKERSFIELD. SO WE ARE ALREADY SEEING PATIENTS THAT COME FROM ALL OVER THE AREA. I THINK, AS YOU KNOW, REALLY THE LARGEST PATIENT VOLUME OF ANY INSTITUTION IN CALIFORNIA.

WHAT WE PROPOSE SPECIFICALLY TO ADDRESS THIS, AND WE TARGET IT WITH SPECIFIC FORMER FELLOWS AND WITH PEOPLE THAT WE'VE COLLABORATED WITH BEFORE, WOULD BE SITES IN NORTHERN CALIFORNIA WHERE THE PATIENTS COULD BE SEEN HERE, BE PART OF OUR CLINICAL TRIALS. WE WOULD HAVE TELEMEDICINE THAT WE CAN ACTUALLY DO IF THERE'S ANY QUESTIONS AND CERTAINLY BE INVOLVED. BUT THAT WOULD REALLY ADDRESS THE NORTHERN CALIFORNIA SPECIFICALLY.

SO WE THINK OUR NETWORK, AND IN PARTICULAR FOR CARDIOVASCULAR DISEASE AND NEUROMUSCULAR

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DISEASE, COULD COVER REALLY ALMOST THE WHOLE STATE. AND WE'VE DONE THIS BEFORE. I DID THIS BEFORE WITH THE STATE OF MINNESOTA WHERE WE COVERED THE ENTIRE STATE WITH A SATELLITE NETWORK, AND IT WAS EXTREMELY SUCCESSFUL.

SUPERVISOR SHEEHY: SO I ALSO -- THIS RELATES TO A LOT OF THINGS. FIRST OF ALL, ONE OF THE THINGS THAT WAS NOTED IN THE REVIEW WAS QUESTIONS ABOUT YOUR SCALE-UP OF GMP MANUFACTURING. AND I WOULD LIKE TO HEAR MORE ABOUT WHAT YOUR MANUFACTURING FACILITY IS LIKE.

AND ONE OF THE OTHER QUESTIONS IS THAT THIS SEEMS TO BE OVERLY RELIANT ON CAPRICOR; AND IF CAPRICOR DOESN'T MAKE IT, WHAT HAPPENS?

AND FINALLY, IN TERMS OF MANUFACTURING, I DO HAVE QUESTIONS ABOUT WHAT YOUR PLAN IS FOR MANUFACTURING WITH SATELLITE SITES. HOW WILL YOU ACTUALLY GET -- WILL YOU BRING THE PATIENTS TO YOU? CERTAINLY SATELLITE SITES ARE FINE FOR RECRUITMENT, BUT I'M NOT AWARE -- IS THERE A PLAN TO HAVE MANUFACTURING FACILITIES IN NORTHERN CALIFORNIA SO THAT YOU CAN ACTUALLY -- CERTAIN TYPES OF CELLS YOU CAN PACK AND SHIP, BUT LOTS OF TYPES OF CELLS YOU ACTUALLY NEED TO BE ABLE TO PROCESS THEM CLOSER TO THE PATIENTS.

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DR. HENRY: AGAIN, THIS IS A COMPLEX QUESTION. AND I WOULD REALLY EMPHASIZE AGAIN THAT CAPRICOR IS AN EXTREMELY SMALL PART OF WHAT WE DO. IN FACT, THE ONLY CLINICAL TRIAL RIGHT NOW THAT WE'RE DOING WITH CAPRICOR IS THE PULMONARY HYPERTENSION TRIAL, AND THAT'S PART OF CIRM. AND ALL OF THE CELLS THAT WE GET FOR ALL THESE OTHER 20 TRIALS ARE NOT INVOLVED IN THEM AT ALL.

SUPERVISOR SHEEHY: SO THEY'RE MANUFACTURED AT CEDARS-SINAI?

DR. HENRY: THEY'RE MANUFACTURED REALLY WHERE THE SPONSOR IS. SO A LOT OF THEM ARE -- A LOT OF THESE -- AND EVERY CELL IS DIFFERENT. SO A LOT OF THEM WE DO THE BONE MARROW LOCALLY AND DO THE LOCAL PROCESSING LOCALLY, WHICH YOU DON'T NEED A MANUFACTURING SITE FOR. SO WHEN YOU DO APHERESIS AND TAKE OUT CD 34 POSITIVE CELLS, THEY'RE READY TO GO AND READY TO GO BACK IN THE PATIENT.

SUPERVISOR SHEEHY: EVERYBODY DOES THAT, BUT DO YOU GENETICALLY MODIFY THEM AT YOUR SITE?

DR. HENRY: SO WHAT WE'RE BUILDING -- WHEN YOU LOOK AT THE WHOLE SPECTRUM OF WHAT WE HAVE, THERE'S A WIDE SPECTRUM OF CELLS. THESE CELLS THAT WE MANUFACTURE AT OUR MANUFACTURING FACILITY ARE ACTUALLY BEING DESIGNED FOR OTHER NEW MODIFIED CELLS

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WITH CLIVE, AND SHOULD BE AVAILABLE, THAT MANUFACTURING SITE SHOULD BE AVAILABLE WITHIN THE NEXT YEAR. SO THAT'S CURRENTLY UNDER WAY FOR THAT SPECIFIC TYPE.

BUT WHEN YOU DO IT, IT'S LIKE EVERY TRIAL THAT YOU DO, AND MOST TRIALS ONCE THEY GET TO A PHASE 2 OR A PHASE 3 TRIAL WHICH WE'RE PARTICIPATING IN, THAT'S DEALT WITH WITH THE FDA. AND THERE'S A PLAN OF HOW THOSE CELLS ARE DELIVERED FOR ALL THE SITES ALL ACROSS THE UNITED STATES. AND, YES, THE SATELLITES CAN BE ACTIVELY INVOLVED IN THAT.

FOR EXAMPLE, IN THE NIH CELL THERAPY NETWORK, WE HAVE SEVEN SITES, AND THE CELLS ARE DELIVERED TO ALL SEVEN SITES.

SUPERVISOR SHEEHY: SO YOU'RE NOT PLANNING ON MANUFACTURING, MODIFYING CELLS ON-SITE? YOU DON'T HAVE THAT CAPACITY FOR THE SINGLE ALS TRIAL WITH THE ADULT STEM CELLS?

DR. HENRY: ABSOLUTELY. WE ARE DEVELOPING A MANUFACTURING FOR THAT, BUT THAT WILL BE A SELECT PART OF OUR PORTFOLIO. TO HAVE A GOOD PORTFOLIO, YOU REALLY NEED TO HAVE A WIDE SPECTRUM OF WHAT YOU ARE DOING, AND THAT WILL BE A SELECT ONE. FOR INSTANCE, THE ALS, THAT'S WHAT THEY'RE DOING WITH THE CURRENT ALS TRIAL.

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SUPERVISOR SHEEHY: I'M FAMILIAR WITH ALL YOUR TRIALS. I'VE SAT IN REVIEWS OF THE TRIALS THAT YOU'VE HAD COME THROUGH CIRM, AND I KNOW THE DIFFERENT CELL TYPES. OKAY.

ARE THERE OTHER QUESTIONS?

MS. WINOKUR: I KNOW ONE DOESN'T THINK OF L.A. AS BEING RURAL, BUT THERE ARE RURAL PARTS OF YOUR AREA. AND I WONDER IF YOU ARE DOING ANYTHING TO REACH OUT TO THAT.

DR. HENRY: SO WE HAVE A VERY ACTIVE PLAN BECAUSE TRANSPORTATION TO CEDARS IS A BIG ISSUE. AND SO WE HAVE A VERY ACTIVE PLAN FOR PATIENTS WHO COME TO OUR CURRENT REGENERATIVE MEDICINE CLINIC. AND WE HAVE PEOPLE THAT COME FROM THREE, FOUR HOURS AWAY FREQUENTLY, AND WE HELP THEM WITH TRANSPORTATION FOR THOSE THAT NEED IT.

AND WE HAVE -- AGAIN, IT'S A VERY WIDE FOOTPRINT. SO FROM PHOENIX AND LAS VEGAS AND SAN DIEGO WE GET ALL THE TIME. EVERY WEEK IN MY CLINIC I SEE PEOPLE FROM A WIDE SPECTRUM.

MS. WINOKUR: DO YOU DO ANYTHING PROACTIVE ABOUT SERVING THOSE AREAS?

DR. HENRY: YES. WHEN SOMEONE APPROACHES US FROM THAT FAR AWAY, WHAT WE FIRST DO IS WE GET ALL THE RECORDS IN, REVIEW ALL THE RECORDS, AND

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DECIDE AHEAD OF TIME, LIKE GIVE THEM YOU'D BE ELIGIBLE FOR THESE THREE OR FOUR OPTIONS. AND THEN WHEN WE MAKE A PLAN FOR THEM TO COME, IN THAT PROCESS WE ASK DO YOU NEED HELP WITH TRANSPORTATION OR NOT. SO IT'S INCLUDED IN PART OF THE PROCESS.

MS. WINOKUR: I'M THINKING OF ISOLATED AREAS LIKE BISHOP, FOR EXAMPLE.

DR. HENRY: WE SEE PEOPLE FROM ALL THOSE AREAS ALREADY, AND IT'S PART OF OUR PLAN, WHEN THEY CALL US, TO BUILD IN SO THAT THEY CAN EASILY GET TO CEDARS AND BACK. AND IT'S A VERY IMPORTANT ISSUE.

SUPERVISOR SHEEHY: OTHER QUESTIONS? SO MAYBE WE CAN HEAR FROM THE NEXT APPLICANT, GIL.

DR. SAMBRANO: SO THE NEXT APPLICATION IS 10314. THIS IS "ALPHA STEM CELL CLINIC FOR NORTHERN AND CENTRAL CALIFORNIA" IS ITS TITLE. THIS ONE RECEIVED A UNANIMOUS SCORE OF 1 FROM 13 MEMBERS. AND DR. JAN NOLTA WILL BE PRESENTING FOR THIS GROUP.

DR. NOLTA: THANK YOU. I'M DELIGHTED TO BE HERE. WE ACTUALLY HAVE THREE PRESENTERS, BUT WE'RE GOING TO GO VERY QUICKLY. OUR PI, DR. MEHRDAD ABEDI, IS HERE, AND ALSO JIM KOVACH. AND WE HAVE EIGHT WONDERFUL PEOPLE FROM MY TEAM TO HELP ANSWER QUESTIONS.

SO WE THANK CIRM FOR ALL THAT YOU'VE DONE

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WITH US. WE SHARE YOUR MISSION OF BRINGING STEM CELLS TO PATIENTS WHO NEED BETTER TREATMENTS AND BETTER OPTIONS. WE HAVE HAD FUNDING FROM CIRM TO RENOVATE OUR INSTITUTE FOR REGENERATIVE CURES, WHICH IS RIGHT NEXT TO OUR CLINICAL TRANSLATIONAL SCIENCE CENTER. WE COLLABORATE WITH THEM VERY CLOSELY. WE HAVE A FABULOUS GMP FACILITY. I'LL HAVE A COUPLE OF SLIDES ON THAT. AND WE HAVE SEVERAL TRAINING PROGRAMS, HIGH SCHOOL AND THE LOCAL STATE COLLEGE, THAT WE ARE VERY PROUD OF AND VERY CLOSE TO MY HEART TO TRAIN ALL OF THESE BUDDING DOCTORS AND SCIENTISTS THAT ARE VERY COMMITTED TO STEM CELLS AND REGENERATIVE MEDICINE.

WE HAVE 24 STEM CELL AND REGENERATIVE MEDICINE CLINICAL TRIALS ONGOING OR RECENTLY COMPLETED AND OVER 20 IN THE PIPELINE. WE ARE VERY, VERY EXCITED TO HAVE THE CHANCE TO BE IN THIS ALPHA CLINIC NETWORK, TO HELP RECRUIT ADDITIONAL PATIENTS FOR OUR OWN TRIALS. WE'RE EXTREMELY SERVICE ORIENTED. WE WANT TO HELP RECRUIT OUR NORTHERN CALIFORNIA PATIENTS TO THE TRIALS THAT ARE ONGOING ELSEWHERE IN CALIFORNIA AND THROUGH THE NETWORK.

THIS GOOD MANUFACTURING PRACTICE FACILITY IS VERY WELL ESTABLISHED NOW. IT WAS FUNDED BY THE CIRM MAJOR FACILITIES GRANT. SO IT'S REALLY YOUR

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GMP FACILITY. WE RUN IT ON A FEE-FOR-SERVICE BASIS. DOESN'T HAVE TO BE OUR IDEA OR OUR TRIAL. WE MANUFACTURE CELL AND GENE THERAPY PRODUCTS, VECTORS, IMMUNOTHERAPY FOR MANY ACADEMIC AND INDUSTRY PARTNERS THROUGH A FEE-FOR-SERVICE BASIS.

THIS IS A SUBSET OF SOME OF THE PRODUCTS THAT ARE CURRENTLY MANUFACTURED. I DON'T HAVE TIME TO GO THROUGH THEM, BUT WE ARE DOING THE FULL SPECTRUM OF CELL TYPES AND GENE MODIFICATION.

AND THIS IS JUST TO SHOW WHERE ON THE SLIDE -- WE ARE LOCATED ON OUR MEDICAL CAMPUS IN SACRAMENTO. THIS IS OUR INSTITUTE FOR REGENERATIVE CURES. WE PROCESS THE PRODUCTS, SEND THEM OUT TO OUR LOCAL SITES WHERE THE STEM CELL CLINICAL TRIALS ARE ONGOING, AND OUR PROPOSED ALPHA CLINIC WILL BE UP HERE IN THE CYPRESS BUILDING WHERE OUR CTSC CLINICAL SITE CURRENTLY IS. WITH THAT, I WILL TURN IT TO OUR PI, DR. ABEDI.

DR. ABEDI: SO REALLY QUICK. SO THIS IS OUR MISSION. I'M NOT GOING TO GO THROUGH THAT, BUT WE HAVE A PLEDGE TO GET THIS DONE ON THE PROPOSED TIMELINE AND REACHING THE FOUR ENROLLMENTS THERE. THIS IS OUR GROUP. WE ARE VERY PROUD OF THAT. THERE IS A VERY GOOD COLLABORATION WITH OUR GMP FACILITY GOING TO ADMINISTRATIVE TEAM TO GET THE

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TRIALS THROUGH IND AND IRB AND EVENTUALLY GETTING TO CLINICAL TRIAL, OUR REGULATORY PART OF IT. AND CTSC IS ARGUABLY ONE OF THE BEST IN THE COUNTRY, AND WE ARE VERY PROUD OF THAT. AND COLLABORATION WITH THE OTHER TEAMS WILL BE VERY INSTRUMENTAL HERE.

HOW WE GOING TO DO IT? WE HAVE A VERY ACTIVE GMP. WE ARE PROPOSING IT'S GOING TO ACTUALLY INCREASE THE CAPACITY HERE. WE ARE PROVIDING FAST SERVICE FOR GETTING IND AND IRB PROTOCOLS GOING. OUR INFUSION CENTER WILL BE INSTRUMENTAL HERE, AND OUR TRAINING AND AS WELL AS THE TELEMEDICINE PROGRAM WILL BE VERY IMPORTANT.

THIS IS MY WISH LIST. THIS IS SOMETHING WE ALREADY HAVE IN PLACE, BUT WE JUST WANT TO MOVE IT NOW. IT'S VERY UNDERUTILIZED. WE'RE GOING TO USE THIS AS OUR ALPHA CLINIC. WE'RE GOING TO HAVE EVERYTHING IN ONE PLACE. AGAIN, DOING THIS FOR 20 YEARS, DOING STEM CELL OR CELL THERAPY CLINICAL TRIALS FOR 20 YEARS, WE ALWAYS HAVE BEEN STRUGGLING WITH GETTING EVERYTHING DONE IN ONE PLACE, AND THIS IS GOING TO GIVE US AN OPPORTUNITY FROM STORAGE PLACE WITH LIQUID NITROGEN TO EQUIPMENT TO DO THE INFUSIONS, MONITORING THE PATIENTS DURING INFUSIONS, AND THEN THE SPECIMEN SAMPLING AND STORAGE AND ALL OF THOSE.

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WHAT WE GOING TO BRING TO ALPHA CLINIC IS THE DIVERSITY MORE THAN ANYTHING ELSE. WE HAVE A VERY DIVERSE POPULATION OF PATIENTS THERE, AND ALSO WE HAVE A VERY DIVERSE PORTFOLIO OF STEM CELL REGENERATIVE-SPECIFIC FOCUSED TRIALS THERE. AND THIS IS OUR CATCHMENT AREA. THERE'S 33 COUNTIES THERE, AND IT'S HUGE, BUT WE ARE ALSO REACHING EVERYWHERE ELSE. OUR GMP FACILITY IS PRODUCING FACTORS AND PRODUCTS FOR UC SAN DIEGO. WE'RE WORKING WITH L.A. AREA, WE'RE WORKING WITH ALL THE WAY DOWN TO L.A. AREA.

THIS IS SOMETHING, AGAIN, WE ARE VERY PROUD OF. THIS IS OUR TELEHEALTH. IT'S SOMETHING THAT'S ALREADY ESTABLISHED, IS ALREADY THERE. THEY ARE DOING -- JUST RECENTLY THEY PUBLISHED 22,000 VIDEO-BASED CONSULTATIONS IN 150 CLINICS ACROSS 56 OUT OF THE 58 COUNTIES IN CALIFORNIA THROUGH ALL THE SUBSPECIALTIES. EVERYTHING YOU CAN IMAGINE. THEY'RE DOING -- ALREADY IT IS ESTABLISHED AND IS WORKING. AND THEY ALREADY HAVE CLINICAL TRIALS WITH ALS, WITH PSYCHIATRY DOING SOME KIND OF NONSTEM CELLS, BUT WE ARE ALREADY DOING CLINICAL TRIAL WITH THAT SYSTEM. SO IT'S ALREADY ESTABLISHED, IT'S ALREADY WORKING, MULTIPLE PUBLICATIONS THERE.

AND THIS IS JUST THE FIRST EXAMPLE OF STEM

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CELL-BASED OR CELL THERAPY-BASED CLINICAL TRIALS THAT WE ARE STARTING TO DO THAT THROUGH OUR TELEMEDICINE PROGRAM.

DR. KOVACH: I'M HAPPY TO ANSWER ANY QUESTIONS ABOUT THE SUSTAINABILITY, WHICH IS AN IMPORTANT PART OF THE ALPHA CLINIC AS WELL. SO THIS IS FROM PERKINS & WILL WHO WE COMMISSIONED TO PROVIDE US INSIGHT INTO WHAT COULD GO ON OUR NEARLY 30 ACRES ON OUR CAMPUS. AND THIS IS PART OF THE VISION, TO ADD TWO MILLION SQUARE FEET, AND WE HOPE TO HAVE MUCH OF THAT ALLOWING US TO EXPAND OUR GMP TO INCREASE TRANSLATIONAL RESEARCH BASE FOR REGENERATIVE MEDICINE. THANKS.

SUPERVISOR SHEEHY: SO DO ANY MEMBERS HAVE QUESTIONS?

CHAIRMAN THOMAS: DR. NOLTA, I'LL ASK THE SAME QUESTION OF YOU ON THE ISSUE OF RECRUITING BEST-IN-CLASS TRIALS. WHAT IS YOUR STRATEGY CURRENTLY? AND WHAT WOULD IT BE IF YOU WERE SO DESIGNATED AS AN ALPHA CLINIC?

DR. NOLTA: GOOD QUESTION. WE ARE REALLY BUSY. I THINK THROUGH OUR GMP FACILITY WE HAVE COMPANIES AND PEOPLE INTERESTED IN CLINICAL TRIALS COMING TO US, NEW MEETINGS EVERY WEEK. THAT'S OUR PIPELINE FOR RECRUITMENT. WE WOULD BRING THAT TO

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THE ALPHA CLINIC FOR CONSULTATION, SEE WHERE THEY MIGHT BEST MANUFACTURE, WHERE THEY MIGHT BEST RECRUIT THE PATIENTS. WE CAN HELP RECRUIT PATIENTS FOR THE ONGOING ALPHA CLINIC CLINICAL TRIALS THROUGH OUR TELEHEALTH. WE IMAGINE THAT IF SOMEBODY -- WE'VE ALREADY BEEN TALKING TO DR. ZAIA AT CITY OF HOPE. OF COURSE, IF SOMEONE WAS TREATED DOWN THERE, THEY COULD RETURN HOME TO NORTHERN CALIFORNIA AND BE SEEN THROUGH TELEMEDICINE.

SO FOR THE NEW PIPELINE COMING IN, THEY'RE COMING TO US KIND OF REALLY, REALLY STEADILY.

CHAIRMAN THOMAS: THANK YOU. DO YOU ACTIVELY TRY TO RECRUIT, OR DO YOU WAIT MORE FOR PEOPLE TO COME TO YOU? OBVIOUSLY THE GMP FACILITY IS A VERY WELL-KNOWN ASSET.

DR. NOLTA: WE DO RECRUIT. WE GO TO THE NATIONAL MEETINGS AND PUT OUT OUR FLIERS AND BROCHURES AND TALK TO PEOPLE WHO MIGHT BE INTERESTED IN BRINGING NEW TRIALS TO US.

I FORGOT TO MENTION WE HAVE 18 CORES THAT OUR PROGRAM RUNS FOR IND-ENABLING STUDIES. WE HAVE A LARGE ANIMAL SURGERY CORE, WE HAVE IMMUNE DEFICIENT MICE, HUMANIZED MICE, VECTOR CORE, AND WE ADVERTISE THOSE AS WELL. THOSE ARE RUN THROUGH FEE-FOR-SERVICE. THEY'RE ALL BREAKING EVEN OR JUST

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ABOUT TO, AND THAT'S HOW WE KEEP OUR REALLY SKILLED PEOPLE IN THE PROGRAM. ALL THE GRANTS GO UP AND DOWN. AND THEY WORK ON CLINICAL TRIALS. AS I MENTIONED, IT DOESN'T HAVE TO BE OUR PRODUCT. WE JUST REALLY WANT TO HELP GET THESE CURES TO THE PATIENTS.

CHAIRMAN THOMAS: THANK YOU.

SUPERVISOR SHEEHY: OTHER QUESTIONS?

DR. PADILLA: I'M FROM THE FRESNO AREA, SO I WAS REALLY PLEASED TO SEE THE TELEMEDICINE COMPONENT. WHEN I SAW YOUR OUTREACH MAP, THE REAL CENTRAL VALLEY AS FAR AS FRESNO, KINGS, TULARE WAS NOT LISTED. I'M CURIOUS AS TO HOW -- THERE'S SUCH A NEED FOR RECRUITMENT FOR PATIENTS IN THAT AREA -- HOW YOU MIGHT OR MIGHT NOT BE ABLE TO REACH THAT SEGMENT OF THE POPULATION.

DR. NOLTA: THAT'S A REALLY GOOD POINT. THROUGH OUR UMBILICAL CORD BLOOD COLLECTION PROGRAM THAT WE'RE RUNNING THROUGHOUT CALIFORNIA, WE DO HAVE A SITE IN FRESNO. SO WE ARE TALKING TO THEM, BUT MAYBE TODD STOLTZ MIGHT WANT TO TALK ABOUT THE TELEHEALTH.

DR. STOLTZ: I'M TODD STOLTZ. I WORK IN THE CENTER FOR HEALTH AND TECHNOLOGY, WHICH IS OUR TELEMEDICINE PROGRAM. WE HAVE SEVERAL ACTIVE

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RELATIONSHIPS DOWN IN THE FRESNO AREA. OUR CANCER CARE NETWORK HAS A JOINT VENTURE WITH THE CANCER CENTER DOWN THERE. WE HAVE SEVERAL RELATIONSHIPS WITH HOSPITALS THROUGH OUR REGIONAL AFFILIATIONS GROUP TO HELP FACILITATE PARTNERSHIPS.

SO AS THE ALPHA CLINIC STEM CELL TRIALS PROGRAM GETS UNDER WAY AND STARTED GROWING, WE WOULD BE ABLE TO START TO INFORM THOSE PARTNERS OF THE OPPORTUNITIES THAT ARE AVAILABLE THROUGH THESE TRIALS TO HELP RECRUIT PATIENTS AND RETAIN THEM IN TRIALS BY KEEPING THEM IN THEIR COMMUNITY.

DR. PADILLA: THERE'S GREAT ONLY BECAUSE THAT CENTRAL AREA SEEMS TO GET LOST IN THE MILIEU. IT'S NOT NECESSARILY NORTHERN, AND IT'S NOT NECESSARILY SOUTHERN CALIFORNIA.

DR. STOLTZ: ABSOLUTELY. WE HAVE A CANCER CLINICAL TRIAL GOING ON REACHING OUT TO HISPANIC POPULATIONS IN THAT COMMUNITY TO HELP RECRUIT THEM INTO SOME OF THESE CANCER TRIALS, WHICH HISPANICS ARE UNDERREPRESENTED IN THOSE TRIALS. IT'S A HUGE, HUGE AREA OF NEED.

DR. PADILLA: THANK YOU.

DR. WUN: I'M TED WUN. I'M THE PI OF THE CTSC. I'M ALSO DIVISION CHIEF FOR HEMATOLOGY/ONCOLOGY AT OUR CANCER CENTER. AND I'D

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LIKE TO SPEAK TO THAT SPECIFICALLY. WE DO HAVE TARGETED OUTREACH. YOU'RE EXACTLY RIGHT. I ACTUALLY WAS BORN AND RAISED IN THAT PART OF CALIFORNIA. AND THERE'S A CRITICAL NEED FOR A LOT OF THE THINGS THAT YOU ARE SAYING.

AND SO AS TODD MENTIONED, WE HAVE A CANCER CARE NETWORK THAT GOES BEYOND THAT. WE HAVE AFFILIATIONS AND GROWING AFFILIATIONS WITH SOME OF THE COMMUNITY HOSPITALS. WE HAVE ACTUALLY A SPECIFIC TRAINING PROGRAM, A MEDICAL STUDENT TRAINING PROGRAM, TO PLACE PRIMARY CARE PHYSICIANS IN THE CENTRAL VALLEY. SO I THINK WE RECOGNIZE THAT. WE BELIEVE THAT'S PART OF OUR MISSION AS THE MEDICAL SCHOOL IN THE CENTRAL VALLEY. SO I THINK THIS WOULD ACTUALLY AUGMENT FURTHER THE ABILITY TO PROVIDE CARE FOR THEM.

DR. DEAS: THIS IS A FOLLOW-UP AND MORE OUTREACH ACTUALLY. I NOTICE THAT YOU HAD ON YOUR SLIDE THE NATIVE AMERICAN CLINIC. WHAT HAS BEEN THE YIELD OF PARTICIPATION FROM THAT AREA?

DR. STOLTZ: SO SPECIFICALLY WITHIN MY PURVIEW, WE HAVE SPECIFICALLY OUTREACH TO THAT POPULATION MAINLY IN THE NORTHERN PART OF THE VALLEY. ONE OF MY RECENTLY RETIRED FACULTY HAD A CANCER PROGRAM ESSENTIALLY FOCUSING ON SCREENING,

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MAMMOGRAPHY, COLONOSCOPY SCREENING BECAUSE THERE'S A LOT OF MISUNDERSTANDING OR DIFFERENT ORIENTATION TO THAT. AND SO I THINK IT'S BEEN STRONGEST IN THOSE.

WE ALSO HAVE, SINCE SHE RETIRED, OUR DISPARITIES PROGRAM WITHIN THE CANCER CENTER AS WELL AS NOW THE CTSC HAS INCORPORATED PROGRAMS IN THE NATIVE AMERICAN POPULATION.

DR. ABEDI: AS I MENTIONED, SOME OF OUR TELEMEDICINE SITES ARE IN THE NATIVE AMERICAN AREAS. AND THE IDEA IS BASICALLY WITH TELEMEDICINE WE ARE NOT GOING TO TAKE THE CELLS OVER THERE AND GIVE THE CELLS. BUT MANY OF THESE CLINICAL TRIALS, THE IDEA IS THAT BASICALLY YOU GET THE PATIENTS, YOU SCREEN THE PATIENTS, AND EVEN SCREENING WE ARE LOOKING AT THE TELEMEDICINE IDEA AS USING THE SCREEN SO YOU DON'T HAVE TO BRING ALL THE PATIENTS HERE AND HAVE 50-PERCENT FAILURE RATE AND SENDING BACK. EVEN FOR THE PRESCREENING, WE ARE INTERESTED IN TELEMEDICINE AS THAT, BUT THEN WE'RE GOING TO GET THE PATIENTS TO OUR CENTER ONCE THEY ARE PRESCREENED, THE STEM CELL APPLICATION, WHATEVER THAT STEM CELL APPLICATION IS INTENDED TO BE DONE WILL BE DONE, AND THEN THE PATIENTS CAN GO BACK. THE IDEA IS THE FOLLOW-UP.

MANY OF MY PATIENTS ARE COMING FROM, FOR EXAMPLE, BEDFORD, OREGON. WE WANT TO PUT THEM IN

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CLINICAL TRIALS, AND THEY SAY, "NO, I CANNOT COME EVERY WEEK TO HAVE A FOLLOW-UP THERE JUST TO SEE HOW I'M DOING OR DO I HAVE A RASH OR NOT." SO THAT'S THE IDEA OF TELEMEDICINE, APPLYING THAT. WE CAN SIT THERE IN A LOCAL PLACE AND HAVE A FOLLOW-UP, QUICK FOLLOW-UP, EVERY WEEK OR EVERY TWO WEEKS. AND IF THEY HAVE TO COME BACK, THEN WE WILL COME BACK.

SUPERVISOR SHEEHY: OTHER QUESTIONS? SO, DR. SAMBRANO, SHALL WE BRING ON THE NEXT APPLICANT?

DR. SAMBRANO: YES, ABSOLUTELY. SO THE FINAL APPLICATION IS 10361 TITLED "CIRM ALPHA STEM CELL CLINIC." AGAIN, THIS APPLICANT RECEIVED A SCORE OF 1. THERE WERE NINE VOTES GIVING IT A SCORE OF 1 AND SIX VOTES GIVING IT A SCORE OF 2. AND DR. MARK WALTERS WILL BE PRESENTING FOR THIS GROUP.

DR. WALTERS: I'D LIKE TO THANK THE COMMITTEE FOR INVITING US TO PRESENT OUR PROPOSAL. I'M HERE ON BEHALF OF THE UC SAN FRANCISCO ALPHA STEM CELL CLINIC PROPOSAL AS A PROJECT DIRECTOR. STEERING COMMITTEE MEMBERS ARE LISTED, AND IN THE ROOM TODAY MICHAEL MATTHAY, WHO IS CO-CHAIR OF THE STEERING COMMITTEE, IS PRESENT, AS ARE DR. KATHLEEN LIU, DR. HERMISTON, AND I'M PLEASED TO HAVE ALSO DR. JENNIFER GRANDIS, WHO'S THE PI OF OUR CTSI AND ALSO HAS AGREED TO CHAIR THE EXTERNAL ADVISORY BOARD FOR

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THE UCSF ALPHA STEM CELL CLINIC.

A BIT OF BACKGROUND ABOUT OUR PROPOSAL. UCSF IS A LARGE BIOMEDICAL AND HEALTH SCIENCE EDUCATION CENTER WITH AN ANNUAL BUDGET THAT EXCEEDS $3 BILLION. CURRENTLY WE RANK FOURTH IN CIRM AWARDS THAT TOTAL 142.6 MILLION AS SHOWN IN THE SLIDE. TOGETHER THESE UNDERLIE A STRONG TRACK RECORD AND COMMITMENT TO STEM CELL AND REGENERATIVE MEDICINE RESEARCH, BUT THE PURPOSE OF THIS PROPOSAL WAS REALLY TO ADDRESS GAPS IN OUR PRODUCTIVITY TO HELP US LIVE UP TO EXPECTATIONS ABOUT WHAT WE CAN ACHIEVE.

AND WHAT WE ATTEMPTED TO ADDRESS ARE PROBLEMS RELATED TO ACCESS TO RESOURCES THAT CAN BE OPAQUE AND TIME CONSUMING. AS CLINICAL TRANSLATIONAL PRINCIPAL INVESTIGATORS DEVELOP THEIR PROJECTS, THERE'S A TENDENCY TO DO THIS IN ISOLATED SILOS, SO DUPLICATION OF EFFORT CAN BE AN ISSUE RELATED TO EFFICIENCIES. AND THEN AN INTEGRATED INFRASTRUCTURE FOR CELL THERAPIES IN PARTICULAR IS LACKING. SO THESE ARE THE GAPS THAT WE AIM TO ADDRESS IN OUR PROPOSAL.

THE PRINCIPAL AIMS, OVERARCHING GOALS, ARE LISTED HERE. THEY'RE, I THINK, CONSISTENT WITH THE GOALS OF THE ALPHA STEM CELL CLINICAL NETWORK TO

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EXPAND CLINICAL ACTIVITY AND CELLULAR THERAPIES AT UC SAN FRANCISCO, LIKE ACCELERATING THE TEMPO OF PRE-AWARD PLANNING WITH TRIAL SPONSORS, CLINICAL TRIAL ACTIVATION, PATIENT ACCRUAL, IDENTIFYING PATIENTS WHERE THEY LIVE AND BRINGING THEM TO THE CLINICAL TRIAL CENTER, AND COMPLETING THE TRIAL ON TIME, EXPANDING PARTICIPATION BY UNDERSERVED POPULATIONS, WHICH IS AN EMPHASIS OF OUR APPLICATION IN PARTICULAR AT UCSF AND ALPHA CLINIC NETWORK, AND TO ESTABLISH DISEASE TEAMS THAT PROMOTE PARTICIPATION AT UCSF AND ALSO THE NETWORK TRIALS MORE BROADLY.

SO THE ORGANIZATION -- WE DECIDED TO FOCUS ON THREE CLINICAL THEMES WHERE WE HAVE PARTICULAR EXPERTISE AND TRACK RECORD OF PRODUCTIVITY. THESE FOCUS ON HEREDITARY, HEMATOLOGICAL, AND IMMUNOLOGICAL DISORDERS, ANTI-INFLAMMATORY AND IMMUNE-MODULATED CELLULAR THERAPIES, AND CELL-BASED THERAPY FOR CRITICALLY ILL PATIENTS.

THE OTHER THING THAT'S IMPORTANT TO EMPHASIZE IS THAT OUR ALPHA CLINIC OPERATIONS AT UC SAN FRANCISCO WILL FOCUS ON CLINICAL SITES IN SAN FRANCISCO, AT THE ZUCKERBERG SAN FRANCISCO GENERAL HOSPITAL, AT MISSION BAY WHERE THERE'S A CHILDREN'S HOSPITAL AND CANCER CENTER, AT THE MAIN CAMPUS AT

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PARNASSUS IN THE CITY, AND THEN OUR CLINICAL OPERATIONS AT BENIOFF CHILDREN'S HOSPITAL OAKLAND IN OAKLAND.

AND OUR PROPOSAL FOCUSED FIRST ON ACCELERATING THE INITIATION OF THE ALPHA STEM CELL CLINIC AT UC SAN FRANCISCO BY USING EXISTING CTSI RESOURCES THAT WOULD HELP US BEGIN AND THEN TRANSITION TO INDEPENDENCE IN THE COURSE OF THE FOUR-YEAR GRANT AWARD.

I'D LIKE TO SPEND TWO MINUTES ON THE CRITIQUES THAT I THOUGHT WERE MOST IMPORTANT. THE FIRST IS THAT THE CTSA IS NOT A LONG-TERM RESOURCE THAT CAN BE RELIED UPON NECESSARILY FOR SUSTAINING THE OPERATION. AGAIN, I'D EMPHASIZE THAT WE'RE USING THIS AS A START-UP TO ACCELERATE LAUNCHING THE OPERATION, BUT WE HAVE OTHER SELF-SUSTAINING IDEAS TO EXTEND THE ALPHA STEM CELL CLINIC AT UCSF.

THE FELLOWSHIP TRAINING WAS MENTIONED AS AN AREA THAT PERHAPS WASN'T WELL DEVELOPED IN THE APPLICATION. I LISTED HERE THREE EXISTING FELLOWSHIP TRAINING EXAMPLES AT UC SAN FRANCISCO AND FOCUSED ON THE CATALYST AWARD PROGRAM IN PARTICULAR BECAUSE WE WILL ADAPT THAT FOR TRAINING THAT WOULD INCLUDE IND PREPARATION, CMC, AND CELLULAR DRUG PRODUCT DEVELOPMENT, PROTOCOL DEVELOPMENT TRAINING

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MODULE, AND THEN HOW TO RECRUIT PATIENTS FROM REMOTE SITES.

WE HAVE TWO GMP'S CURRENTLY AT UCSF, A LARGE ONE AT THE MISSION CENTER LOCATION, 4500 SQUARE FEET, AND A SMALLER ONE AT THE MISSION BAY PEDIATRIC STEM CELL THERAPY LAB. AND THE QUESTION WAS DO WE REALLY NEED TWO LABS. THE SCOPE OF SERVICES ARE QUITE DIFFERENT AT THE TWO SITES. SO WE DO JUSTIFY IT ON THAT MEANS. THE ONE AT MISSION BAY FOCUSED ON A STEM CELL TRANSPLANT PROGRAM, A CLINICAL PROGRAM, BUT ALSO GENE THERAPY FOR VARIOUS HEREDITARY DISORDERS, AND THE ONE AT MISSION CENTER FOCUSED ON T REGULATORY THERAPIES.

THERE WAS A QUESTION ABOUT HOW WOULD WE INTEGRATE ACROSS SITES. I'VE SHOWN A PICTURE OF A TELEMEDICINE CONFERENCE THAT WE HAD JUST THIS WEEK ILLUSTRATING PARTICIPANTS AT THE PARNASSUS, MISSION BAY, AND OAKLAND SITES. AND THEN TIPPI MACKENZIE, WHO'S A MEMBER OF THE STEERING COMMITTEE, NOT HERE TODAY, BUT HAS VERY NICELY LAUNCHED A PROJECT THAT RELIES UPON EXPERTISE IN HEMOGLOBIN DISORDERS IN OAKLAND, HER OWN IMMUNE TOLERANCE WORK AT PARNASSUS, AND THEN THE FETAL SURGICAL TEAM WHERE IN UTERO TRANSPLANTS FOR ALPHA THALASSEMIA MAJOR WILL BE CONDUCTED AT THE MISSION BAY CAMPUS.

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MICHAEL MATTHAY WHO'S HERE LEADING THE MCS TRIALS FOR ARDS HAS PLANS TO EXPAND THOSE TRIALS FOR RESPIRATORY DISTRESS SYNDROME AT UCLA AND AT CHILDREN'S HOSPITAL, AND WE HAVE STEERING COMMITTEE EXPERTISE ACROSS THESE DISORDERS.

THE BUSINESS PLAN SHOULD INCLUDE CAR T TRIALS, AND IN THE INTERIM MICHELLE HERMISTON, WHO'S HERE, HAS ACTIVATED SEVERAL INDUSTRY-SPONSORED TRIALS, AND OUR OWN COLLABORATION WITH SEATTLE WEST COAST CONSORTIUM FOR CAR T THERAPY IN OAKLAND, AND THEN AN EXPANDED ACCOUNTABLE CARE ORGANIZATION HAS OCCURRED IN THE INTERIM CALLED CANOPY HEALTH, WHICH IS NOW THE SIXTH LARGEST HEALTH SYSTEM IN THE COUNTRY.

SO SUMMARY IS HERE. WE HAVE BROAD AND UNIQUE RESOURCES. THE THEMES WOULD BE UNIQUE TO THE DISORDERS THAT I'VE PRESENTED WITH EXPERIENCED INVESTIGATORS. THANKS VERY MUCH.

SUPERVISOR SHEEHY: SO ANY QUESTIONS?

CHAIRMAN THOMAS: LIKE THE SAME QUESTION ON RECRUITING, AND I WOULD ADD TO IT, JUST TO SORT OF INFORM THE BOARD, HOW DO YOU GO ABOUT TRYING TO DISTINGUISH UCSF IN GENERAL AS THE PLACE TO DO CLINICAL TRIALS AS OPPOSED TO THE MANY OTHER QUALIFIED PLACES THAT ARE CONDUCTING TRIALS, LET'S

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SAY, IN NORTHERN CALIFORNIA?

DR. WALTERS: SO I'M GOING TO ANSWER THAT IN PARTICULAR WITH REGARD TO THE HEMOGLOBIN DISORDERS BECAUSE THAT'S AN AREA WHERE I HAVE EXPERTISE, AND WE ARE A SITE OF EXCELLENCE FOR THOSE DISEASES. AND SO HAVE WORKED OUT OUTREACH TO BRING IN THE PATIENTS AND THEN TO CONDUCT THE TRIALS WITH TRAINED PHYSICIANS AND NURSES WHO ARE EXPERT IN THE DISORDERS.

I THINK THE SAME CAN BE SAID OF RESPIRATORY DISTRESS SYNDROME AND THE TREATMENT WITH MESENCHYMAL STEM CELL THERAPIES AS WELL. THESE ARE UNIQUE TRIALS SPECIFIC TO CLINICAL PROBLEMS WHERE THERE IS EXPERTISE AT SAN FRANCISCO, UC SAN FRANCISCO.

CHAIRMAN THOMAS: AND GENERALLY ON THE FIRST QUESTION, HOW DO YOU GO ABOUT RECRUITING TRIALS? AND HOW ACTIVE ARE YOU IN GOING OUT AND PURSUING THEM AS OPPOSED TO SORT OF WAITING FOR THEM TO COME TO YOU?

DR. WALTERS: RIGHT. WELL, IT'S A BIT OF DOUBLE-EDGED SWORD. SO WHAT WE FIND IS THAT WE'RE APPROACHED BY MORE SPONSORS THAN WE CAN ACCOMMODATE CURRENTLY WITH THE EXISTING INFRASTRUCTURE. SO BY EXPANDING THE INFRASTRUCTURE THROUGH THE ALPHA STEM

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CELL CLINIC FUNDING, WE WOULD BE ABLE TO ACCOMMODATE MORE OF THOSE ENCOUNTERS AND TRANSLATE THOSE INTO ACTUAL CLINICAL TRIALS.

AS FAR AS HOW WE RECRUIT, IT HAS A LOT TO DO WITH NETWORKING WITHIN THE PARTICULAR DISEASE OR THE TYPE OF DISORDERS TREATED. SO GOING TO NATIONAL MEETINGS, WORKING WITH INVESTIGATORS AT DIFFERENT INSTITUTIONS, PARTICIPATING IN MULTICENTER SITE TRIALS, AND ACTING AS LEADERSHIP IN THOSE MULTICENTER NATIONAL TRIALS ALTOGETHER BRING THE TRIALS TO SAN FRANCISCO.

SUPERVISOR SHEEHY: ARE THERE OTHER QUESTIONS?

DR. STEWARD: AGAIN, THE QUESTION OF GEOGRAPHIC REACH BEYOND THE, WHATEVER, 60-MILE RADIUS OF WHERE WE SIT RIGHT NOW. CAN YOU JUST UNPACK THAT A LITTLE BIT MORE?

DR. WALTERS: SURE. SO ONE OF THE ACTIVE RESEARCH AREAS FUNDED BY THE NATIONAL INSTITUTES OF HEALTH IS AN IMPLEMENTATION SCIENCE PROJECT FOR HEMOGLOBIN DISORDERS. SO WE'VE EMBARKED UPON A LARGE PROJECT USING SOCIAL MEDIA AS A WAY TO ADVERTISE CLINICAL TRIALS THAT ARE PLANNED OR UNDER WAY AND ALSO SOMETHING CALLED ECHO, WHICH WAS DEVELOPED IN NEW MEXICO AS A MEANS OF TELEMEDICINE

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TO INTERACT WITH LOCAL CLINICS WHERE PATIENTS WITH A SPECIFIC RARE DISORDER MIGHT BE RECEIVING THEIR CARE.

SO THE CHALLENGE REALLY IS THAT THE PERSONS WITH THE DISORDERS THAT MIGHT BE ELIGIBLE FOR OUR TRIALS ARE DISPERSED THROUGHOUT THE STATE, AND THE CHALLENGE THAT WE'RE ATTACKING THROUGH THAT PARTICULAR PROJECT IS HOW TO REACH THEM IN INNOVATIVE WAYS. SO GOOGLE AD, FACEBOOK CONTACTS ADS, OTHER MEANS OF OUTREACH THAT WOULD MAKE THE PUBLIC, NOT NECESSARILY IN OUR MEDICAL CENTER, AWARE OF OPPORTUNITIES TO PARTICIPATE IN THESE TRIALS. AND BECAUSE MANY OF THE TRIALS THAT WE ARE DOING INVOLVE RARE DISEASES WITH DISPERSED POPULATIONS, WE'VE GOTTEN QUITE GOOD AT FINDING WHERE THE PATIENTS ARE BOTH DIRECTLY AND THROUGH OUR NETWORK OF REFERRING PHYSICIANS.

DR. STEWARD: CAN I FOLLOW UP ACTUALLY WITH A SPECIFIC QUESTION? WHICH IS I'M TALKING LESS ABOUT RECRUITING THAN REALLY SORT OF, I WOULD SAY, DELIVERY.

DR. MATTHAY: MARK, OF COURSE, IS LOCATED HERE IN OAKLAND; I'M IN SAN FRANCISCO AT UCSF. SO I WANTED TO ADDRESS THE POINT OF OUTREACH. WE HAVE UCSF FRESNO, WHICH WE'RE VERY COMMITTED TO AS PART

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OF OUR CLINICAL TRIALS WITH THE NIH NOW, AND FROM THE MESENCHYMAL STEM CELL PROGRAM, THIS WILL REACH OUT. WE EVEN HAVE AN ARRANGEMENT TO REACH TO UCLA AS WELL AS OAKLAND CHILDREN'S AND SAN FRANCISCO CHILDREN'S.

AND, FOR EXAMPLE, DR. JENNIFER GRANDIS, WHO'S HERE, YOU MIGHT EXPLAIN HOW WE TRY TO PUT TOGETHER ALL THESE DIFFERENT COMPONENTS.

DR. GRANDIS: HELLO, EVERYBODY. THANK YOU FOR HAVING ME. SO UCSF TAKES ITS ROLE AS A PUBLIC INSTITUTION VERY SERIOUSLY, AND WE SEE IT AS OUR MISSION TO BRING FIRST-RATE CLINICAL CARE, INCLUDING CLINICAL RESEARCH, TO THE STATE OF CALIFORNIA. AND ONE OF THE THINGS THAT WE'VE DONE IS BUILD A COMMUNITY ENGAGEMENT PROGRAM FIRST UNDER THE CTSA, BUT IT'S PART OF OUR CHANCELLOR'S OFFICE. AND THIS COMMUNITY ENGAGEMENT PROGRAM CONTINUES TO EXPAND EXPONENTIALLY AS WE ENGAGE WITH THESE PARTNERS, NOT JUST IN THE CITY OF SAN FRANCISCO, NOT JUST IN THE BAY AREA, BUT REALLY THROUGHOUT THE STATE OF CALIFORNIA, AND REALLY THROUGH THE CTSA CONSORTIUM AS TED CAN ATTEND TO.

WE ALSO ARE A MEMBER OF SOMETHING CALLED UC BRAID WHICH LINKS ALL FIVE HEALTH SCIENCE UC'S IN THE STATE OF CALIFORNIA AND INCLUDES STANFORD AS

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WELL. SO WHAT WE SEE, IN TERMS OF OUTREACH, IS DELIVERING INFORMATION. WE'RE BUILDING SOMETHING CALLED A TRIAL FINDER WITH A SUPPLEMENTAL SMALL GRANT FROM NIH TO ACTUALLY PUT IN HUMAN LANGUAGE, PATIENT LANGUAGE, CLINICAL RESEARCH OPPORTUNITIES THROUGHOUT THE CTSA NETWORK IN CALIFORNIA IN AN ACCESSIBLE WAY ONLINE FOR THE CITIZENS OF CALIFORNIA AS WELL AS FOR REFERRING PHYSICIANS. SO IT'S SOMETHING WE TAKE VERY SERIOUSLY AND WOULD LIKE TO SEE THIS STEM CELL CENTER LEVERAGE.

SUPERVISOR SHEEHY: JUST TO THAT POINT, THIS IS THE ONLY APPLICANT FROM THE BAY AREA, WHICH IS THE FIFTH LARGEST METROPOLITAN AREA IN THE COUNTRY. SO JUST SIMPLY SERVING THE GEOGRAPHIC REACH WITHIN THIS LOCALITY -- WELL, I THINK THERE'S A GAP THERE. JUST TO NOTE THAT. NOT TO BE PAROCHIAL FOR THE BAY AREA.

DO WE HAVE OTHER QUESTIONS? UC FRESNO, I THINK, IS -- I THINK UCSF FRESNO, UCSF DIDN'T JUST GET THERE LAST YEAR. I THINK IT'S 40 YEARS. SO THAT DOES SHOW A COMMITMENT TO THE VALLEY.

SO I THINK WE'VE BEEN THROUGH ALL OF THESE. WE HAVE THREE APPLICATIONS BEFORE US. WE HAVE ENOUGH FUNDING FOR TWO BASED ON THE BUDGET. I BELIEVE THE BUDGET THAT WE ARE ALLOCATED FOR THIS

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WAS 16 MILLION. WE CANNOT GO BEYOND THAT BUDGET WITHOUT -- WE HAVE TO HAVE ANOTHER BOARD MEETING WHERE WE AGENDA THAT. SO I LEAVE IT TO MEMBER'S PLEASURE. WE CAN EITHER HAVE A BROAD DISCUSSION OF THREE APPLICATIONS, OR WE CAN TAKE THEM ONE AT A TIME. IS THERE ANY IMPULSE TO EITHER TAKE THEM INDIVIDUALLY FIRST AND VOTE THEM UP AND DOWN, OR WOULD WE RATHER HAVE A BROAD DISCUSSION OF THE MERITS OF ALL THREE? I TAKE ANYBODY'S OPINION ON THAT IF ANYBODY HAS ONE.

DR. STEWARD: I THINK IT MIGHT BE BETTER TO TAKE THEM ONE AT A TIME UNLESS THERE IS SOME PROCEDURAL REASON, SCOTT, TO DO IT OTHERWISE.

MR. TOCHER: NO. PROBABLY ONE AT A TIME IS PROBABLY THE EASIER WAY TO MANAGE IT. I JUST REMIND YOU, ONCE AGAIN, THAT BECAUSE WE HAVE MORE APPLICANTS THAT ARE TO BE CONSIDERED THAN THERE IS FUNDING FOR, IT PRECLUDES FOLKS WHO HAVE A CONFLICT WITH ANY ONE TO PARTICIPATE IN THE VOTE OR DISCUSSION OF ANY OTHER.

SUPERVISOR SHEEHY: BASED ON THAT REASON, I THINK IT MAKES SENSE TO TAKE THEM ONE AT A TIME. THE ORDER IN WHICH I WILL TAKE THEM IS THE ORDER ON WHICH -- BASED ON SCORES FOR NO. 1. SO I WILL START WITH THE ONE THAT HAS THE HIGHEST NO. 1 SCORES.

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DR. STEWARD: SO ARE YOU ASKING FOR A MOTION?

SUPERVISOR SHEEHY: I'M ASKING FOR A MOTION, YES, PLEASE.

DR. STEWARD: SO I WOULD LIKE TO RECOMMEND FUNDING FOR INFR4-10314. I THINK THAT'S THE RIGHT NUMBER. YES, IT IS. THERE IT IS.

DR. DEAS: SECOND.

SUPERVISOR SHEEHY: WE HAVE A SECOND FROM DR. DEAS.

MR. TORRES: SECOND.

SUPERVISOR TORRES: ALSO SENATOR TORRES. DO WE HAVE ANY DISCUSSION?

MR. PANETTA: I'VE JUST GOT A QUESTION FOR YOU. YOU SAID, DID I HEAR YOU CLEARLY, THAT WE HAVE $16 MILLION FOR THIS?

SUPERVISOR SHEEHY: RIGHT. AND THE BUDGETS ARE RUNNING JUST ROUGHLY 7.9 MILLION APIECE.

MR. PANETTA: GREAT. WE DEFINITELY HAVE ENOUGH TO FUND TWO OF THEM THEN?

SUPERVISOR SHEEHY: YES. SO I HAVE A MOTION AND A SECOND. IS THERE FURTHER DISCUSSION AT THE BOARD? ANY PUBLIC COMMENT ON THIS APPLICATION? SO COULD WE CALL THE ROLL PLEASE.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

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DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: YES.

MS. BONNEVILLE: DAVE MARTIN.

DR. MARTIN: AYE.

MS. BONNEVILLE: LAUREN MILLER. ADRIANA PADILLA.

DR. PADILLA: YES.

MS. BONNEVILLE: JOE PANETTA.

MR. PANETTA: YES.

MS. BONNEVILLE: ROBERT QUINT.

DR. QUINT: YES.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: YES.

MS. BONNEVILLE: JEFF SHEEHY.

SUPERVISOR SHEEHY: YES.

MS. BONNEVILLE: OS STEWARD.

DR. STEWARD: YES.

MS. BONNEVILLE: JONATHAN THOMAS.

CHAIRMAN THOMAS: YES.

MS. BONNEVILLE: ART TORRES.

MR. TORRES: AYE.

MS. BONNEVILLE: DIANE WINOKUR.

MS. WINOKUR: YES.

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MS. BONNEVILLE: MOTION CARRIES.

SUPERVISOR SHEEHY: THANK YOU. SO THE NEXT APPLICATION BASED ON SCORES OF ONE. SO DO I HAVE A MOTION ON THIS APPLICATION?

DR. STEWARD: SO I DO RECOMMEND THAT INFR4-10361 BE FUNDED.

SUPERVISOR SHEEHY: DO I HAVE A SECOND?

DR. JUELSGAARD: SECOND.

SUPERVISOR SHEEHY: SECOND FROM MR. JUELSGAARD. DO WE HAVE BOARD DISCUSSION? DO WE HAVE ANY PUBLIC COMMENT? DON REED.

MR. REED: NO ONE CAN ARGUE WITH THE EXCELLENCE OF ALL THREE PRESENTATIONS. BUT IF WE CHOOSE THIS ONE, WE CAN'T GET UCSF. UCSF IS NORTHERN CALIFORNIA, AND IS ALSO -- OH, THIS IS UC. THEN I HUNDRED PERCENT WANT THIS ONE. SORRY. LITTLE CONFUSED THERE.

ALSO, I HAVE MY GOOGLE ALERT SET FOR STEM CELLS, AND IT'S ASTONISHING HOW OFTEN UCSF COMES UP WITH A SPARK OF INSPIRATION. I THINK WE ARE OUTSTANDING, AND WE ARE WELL SERVED BY HAVING THEM.

ALSO, I HAVE TO SAY THREE WORDS FOR OUR NEW PRESIDENT. WELCOME, WELCOME, AND WELCOME.

SUPERVISOR SHEEHY: THANK YOU. ANY OTHER PUBLIC COMMENT? CAN WE CALL THE ROLL.

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MS. BONNEVILLE: ANNE-MARIE DULIEGE.
DAVID HIGGINS.
DR. HIGGINS: YES.
MS. BONNEVILLE: STEVE JUELSGAARD.
MR. JUELSGAARD: YES.
MS. BONNEVILLE: DAVE MARTIN.
DR. MARTIN: YES.
MS. BONNEVILLE: LAUREN MILLER. ADRIANA PADILLA.
DR. PADILLA: YES.
MS. BONNEVILLE: JOE PANETTA.
MR. PANETTA: YES.
MS. BONNEVILLE: ROBERT QUINT.
DR. QUINT: ABSTAIN.
MS. BONNEVILLE: AL ROWLETT.
MR. ROWLETT: YES.
MS. BONNEVILLE: JEFF SHEEHY.
SUPERVISOR SHEEHY: YES.
MS. BONNEVILLE: OS STEWARD.
DR. STEWARD: YES.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.

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MS. WINOKUR: YES.

MS. BONNEVILLE: MOTION CARRIES.

SUPERVISOR SHEEHY: SO I HAVE A QUESTION FOR COUNSEL, AS YOU MIGHT ANTICIPATE. SINCE OUR FUNDS ARE DEPLETED, DO WE NEED TO TAKE ANY ACTION ON THE REMAINING APPLICATION?

MR. TOCHER: WE DO. WE NEED A VOTE TO NOT FUND THE REMAINING APPLICATION BY -- A MOTION BY A MEMBER WHO DOESN'T HAVE A CONFLICT.

SUPERVISOR SHEEHY: OKAY.

DR. JUELSGAARD: SO I MOVE NOT TO FUND ANY REMAINING APPLICATIONS.

SUPERVISOR SHEEHY: DO I HAVE A SECOND?

MR. ROWLETT: I'LL SECOND.

SUPERVISOR SHEEHY: GREAT. ANY BOARD DISCUSSION? ANY PUBLIC COMMENT? CALL THE ROLL PLEASE.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: YES.

MS. BONNEVILLE: DAVE MARTIN.

DR. MARTIN: AYE.

MS. BONNEVILLE: LAUREN MILLER. ADRIANA

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PADILLA.

DR. PADILLA: YES.

MS. BONNEVILLE: JOE PANETTA.

MR. PANETTA: YES.

MS. BONNEVILLE: ROBERT QUINT.

DR. QUINT: NO.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: YES.

MS. BONNEVILLE: JEFF SHEEHY.

SUPERVISOR SHEEHY: YES.

MS. BONNEVILLE: OS STEWARD.

DR. STEWARD: YES.

MS. BONNEVILLE: JONATHAN THOMAS.

CHAIRMAN THOMAS: YES.

MS. BONNEVILLE: ART TORRES.

MR. TORRES: AYE.

MS. BONNEVILLE: DIANE WINOKUR.

MS. WINOKUR: YES.

MS. BONNEVILLE: MOTION CARRIES.

SUPERVISOR SHEEHY: THANK YOU. I THINK THE NEXT ITEM IS THE CLINICAL APPLICATIONS, BUT I WONDER IF IT MIGHT BE USEFUL TO HAVE A BREAK. WE'VE BEEN AT THIS FOR TWO HOURS. I KNOW THAT -- FIVE MINUTES. SO WE'LL BREAK FOR FIVE MINUTES AND COME BACK AND CONSIDER THE CLIN APPLICATIONS.

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(A RECESS WAS TAKEN.)

CHAIRMAN THOMAS: COULD EVERYBODY PLEASE TAKE YOUR SEATS. WE'RE READY TO RESUME.

SUPERVISOR SHEEHY: WE'RE ALL BACK. SO THE PEOPLE ON THE PHONE? GREAT.

SO, DR. SAMBRANO, WOULD YOU LIKE TO -- I THINK YOU HAVE A PRESENTATION ON THE CLIN APPLICATIONS.

DR. SAMBRANO: YES. THANK YOU, MR. SHEEHY.

ALL RIGHT. SO WE ARE MOVING ON TO THE CLINICAL STAGE PROGRAMS. AS YOU KNOW, WE HAVE THREE PROGRAM ANNOUNCEMENTS THAT ARE SOLICITATIONS FOR APPLICATIONS AT THIS STAGE: CLIN1 THAT SUPPORTS IND-ENABLING WORK, CLIN2 THAT SUPPORTS CLINICAL TRIALS, AND CLIN3 WHICH PROVIDES SUPPLEMENTAL FUNDING TO EXISTING CLINICAL TRIALS UNDER CIRM FUNDING.

TODAY WE'RE GOING TO CONSIDER SIX APPLICATIONS. THERE IS ONE THAT IS RESPONDING TO THE CLIN1 PROGRAM AND FIVE THAT ARE RESPONDING TO THE CLIN2 CLINICAL TRIAL PROGRAM.

REMINDER OF THE SCORING SYSTEM. THIS ONE ALSO USES THE SCORING OF 1, 2, OR 3 WHERE A SCORE OF 1 MEANS IT HAS EXCEPTIONAL MERIT AND WARRANTS

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FUNDING, A SCORE OF 2 MEANS IT NEEDS IMPROVEMENT. SO TYPICALLY APPLICANTS THAT RECEIVE A 2 WILL RESUBMIT WITHIN ABOUT A MONTH IN ORDER TO ADDRESS SPECIFIC COMMENTS FROM REVIEWERS. AND A SCORE OF 3, WHICH MEANS IT'S SUFFICIENTLY FLAWED AND DOES NOT WARRANT FUNDING. AND FOR THOSE, THOSE CANNOT BE RESUBMITTED FOR AT LEAST SIX MONTHS. IT'S A KIND OF GO BACK AND RETHINK THIS.

OKAY. SO THE FIRST APPLICATION UNDER CONSIDERATION IS CLIN2-09574. THIS IS A PHASE 2 CLINICAL TRIAL OF A THERAPY FOR NEUTROPENIA IN AML PATIENTS. SO WHAT THIS IS IT'S AN UMBILICAL CORD BLOOD STEM CELL THERAPY. IT'S ALLOGENEIC, AND IT'S INTENDED TO BE UNIVERSAL, MEANING THAT IT'S ONE THAT DOESN'T HAVE TO BE MATCHED TO THE PATIENTS. THEY ARE GOING TO TEST IT IN A SPECIFIC INDICATION, SO AML PATIENTS WHO ARE UNDERGOING HIGH-DOSE CHEMOTHERAPY WHICH ARE SUBJECT TO NEUTROPENIA OR LOW WHITE BLOOD CELL COUNTS.

THE GOAL IS TO COMPLETE A PHASE 2 CLINICAL TRIAL TO ASSESS SAFETY AND EFFICACY OF THE THERAPY IN THESE PATIENTS. AND THE MAJOR PROPOSED ACTIVITIES INCLUDE THE PREPARATION OF SCALE-UP OF PRODUCT FOR MANUFACTURING, THE ACTUAL GMP MANUFACTURE OF THIS CELL THERAPY, AND THE CONDUCT OF

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THE TRIAL. THE FUNDS REQUESTED ARE 6.9 MILLION. THE APPLICANT IS PROVIDING 16.8 IN CO-FUNDING FOR THIS APPLICATION.

THE GRANTS WORKING GROUP SCORE IS A 1. THE NUMBER OF VOTES IN THE TIER I ARE 12. THERE WERE TWO MEMBERS THAT SCORED THIS AS A 2. THERE WERE NO VOTES IN TIER III. CIRM IS AGREEING WITH THIS RECOMMENDATION BASED ON THE OVERALL PROCESS AND CONDUCT OF THE REVIEW AND, THEREFORE, CONCUR WITH THE GWG RECOMMENDATION TO PROVIDE AN AWARD OF 6.9 MILLION TO THIS APPLICANT.

SUPERVISOR SHEEHY: SO DO I HAVE A MOTION ON THIS APPLICATION?

DR. JUELSGAARD: BEFORE YOU ASK FOR A MOTION, WOULDN'T IT -- FIRST OF ALL, MY UNDERSTANDING IS WE DON'T HAVE THE FUNDING FOR ALL OF THEM. AM I WRONG OR RIGHT ABOUT THAT?

SUPERVISOR SHEEHY: WE HAVE FUNDING FOR ALL OF THEM.

DR. JUELSGAARD: YOU WANT TO DO THESE -- YOU'RE NOT GOING TO DO THEM ALTOGETHER THEN?

SUPERVISOR SHEEHY: NO. WE TAKE THEM ONE AT A TIME.

DR. JUELSGAARD: OKAY.

SUPERVISOR SHEEHY: DO I HAVE A MOTION FOR

THIS APPLICATION?

DR. DEAS: SO MOVED.

MR. TOCHER: DR. DEAS, YOU'RE NOT A MEMBER OF THE APPLICATION REVIEW SUBCOMMITTEE.

DR. STEWARD: SO MOVED.

DR. PRIETO: SECOND.

SUPERVISOR SHEEHY: SECONDED BY DR. PRIETO. ANY BOARD DISCUSSION? ANY PUBLIC COMMENT? CAN WE CALL THE ROLL PLEASE.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: YES.

MS. BONNEVILLE: DAVE MARTIN.

DR. MARTIN: AYE.

MS. BONNEVILLE: LAUREN MILLER. ADRIANA PADILLA.

DR. PADILLA: YES.

MS. BONNEVILLE: JOE PANETTA.

MR. PANETTA: YES.

MS. BONNEVILLE: FRANCISCO PRIETO.

DR. PRIETO: AYE.

MS. BONNEVILLE: ROBERT QUINT.

DR. QUINT: YES.

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MS. BONNEVILLE: AL ROWLETT.
MR. ROWLETT: YES.
MS. BONNEVILLE: JEFF SHEEHY.
SUPERVISOR SHEEHY: YES.
MS. BONNEVILLE: OS STEWARD.
DR. STEWARD: YES.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
SUPERVISOR SHEEHY: THANK YOU. THE NEXT APPLICATION PLEASE, DR. SAMBRANO.
DR. SAMBRANO: THE NEXT APPLICATION IS CLIN2-09672. THIS IS A PHASE 1 TRIAL OF A CELL THERAPY FOR HIGH-RISK TYPE 1 DIABETES. SO THIS IS A HUMAN ESC-DERIVED THERAPY THAT IS DIFFERENTIATED TO PANCREATIC PROGENITORS AND ARE PLACED IN A DELIVERY DEVICE. AND THE TRIAL IS INDICATED IN THIS PARTICULAR CASE FOR HIGH-RISK TYPE 1 DIABETES PATIENTS SUCH AS THOSE THAT HAVE HYPOGLYCEMIA UNAWARENESS OR WHAT'S TERMED BRITTLE DIABETES.
THEIR GOAL IS TO COMPLETE A PHASE 1-2 CLINICAL TRIAL TO ASSESS SAFETY AND EFFICACY OF THE

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CELL THERAPY COMBINATION PRODUCT IN PATIENTS. THE MAJOR PROPOSED ACTIVITIES INCLUDE THE MANUFACTURE AND QUALITY CONTROL OF THE CELL DEVICE PRODUCT, THE LAUNCHING AND CONDUCT OF THIS CLINICAL TRIAL, AND SOME ASSAY DEVELOPMENT ACTIVITIES. THE FUNDS REQUESTED ARE 20 MILLION. THE APPLICANT IS PROVIDING ABOUT 8.6 MILLION IN CO-FUNDING.

THE GWG GAVE THIS A UNANIMOUS SCORE OF 1 WITH 15 MEMBERS VOTING IN THAT TIER. CIRM AGREES WITH THIS RECOMMENDATION IN THE AWARD AMOUNT OF 20 MILLION.

SUPERVISOR SHEEHY: DO WE HAVE A MOTION ON THIS APPLICATION?

MR. TORRES: MOVE IT.

SUPERVISOR SHEEHY: MOVED BY SENATOR TORRES. DO I HAVE A SECOND?

DR. STEWARD: SECOND.

SUPERVISOR SHEEHY: SECOND BY DR. STEWARD. DO WE HAVE ANY BOARD COMMENT?

DR. JUELSGAARD: YES. SO ACTUALLY I'VE TALKED TO DR. MILLAN ABOUT THIS IN ADVANCE SO THAT SHE WOULD BE PREPARED TO ANSWER THE QUESTION.

SO, DR. MILLAN, IT'S MY UNDERSTANDING THAT THE APPLICANT IN THIS CASE HAS BEEN BEFORE US SEVERAL TIMES TO RECEIVE FUNDING FOR ESSENTIALLY THE

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SAME APPROACH TO THERAPY IN THE SAME INDICATION. HOW MUCH HAS THAT APPLICANT BEEN AWARDED TO DATE AND HOW MUCH HAVE THEY SPENT?

DR. MILLAN: I'M GOING TO ASK GABE THOMPSON, WHO'S OUR HEAD OF GRANTS MANAGEMENT, TO RESPOND TO THAT QUESTION.

MR. THOMPSON: SO THIS APPLICANT HAS BEEN AWARDED ABOUT 51.1 MILLION IN FUNDING SINCE INCEPTION ACROSS ABOUT SIX OR SEVEN DIFFERENT AWARDS, TOTALING 51.1 MILLION.

DR. JUELSGAARD: ARE THOSE AWARDS FOR ROUGHLY THE SAME INDICATION, TYPE 1 DIABETES, AND THE SAME MECHANISM FOR TREATING IT?

DR. SAMBRANO: YES, THEY ARE FOR TYPE 1 DIABETES. HOWEVER, THE PATIENT POPULATION THAT'S BEING STUDIED IS ACTUALLY A LITTLE DIFFERENT DEPENDING ON THE TRIAL THAT IS BEING SUPPORTED. SOME OF THOSE STUDIES WERE ACTUALLY THE DEVELOPMENT OF THE DEVICE AND THE CELLS EARLY ON. THIS PARTICULAR PROPOSAL IS FOR A NEW DEVICE, THE SAME CELLS, BUT THE DEVICE IS A SECOND GENERATION PRODUCT THAT IS NOW BEING TESTED.

DR. JUELSGAARD: ALL RIGHT. SO THE REQUEST THIS TIME IS FOR $20 MILLION AND WE'RE STILL BACK IN PHASE 1, VERY EARLY IN THE CLINICAL PROCESS.

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IF WE APPROVE THIS, THEN, ADDING THE 20 TO THE 50 IS 70 MILLION, WHICH IS CLOSE TO TWO AND A HALF PERCENT OF OUR $2.8 BILLION BUDGET. HAS ANYBODY ELSE BEEN AWARDED THIS MUCH MONEY FOR A PARTICULAR INDICATION USING A MECHANISM FOR TREATING IT; THAT IS, A DEVICE-ORIENTED MECHANISM? IS ANYBODY AWARE OF ANYBODY RECEIVING ANY MORE MONEY THAN THAT?

DR. SAMBRANO: WE DON'T HAVE A SINGLE APPLICANT THAT HAS RECEIVED THAT AMOUNT OF FUNDS FROM CIRM.

DR. JUELSGAARD: VERY GOOD. THANK YOU.

SUPERVISOR SHEEHY: FURTHER BOARD DISCUSSION? ANY PUBLIC COMMENT?

MR. REED: JUST THAT THE FACT THAT WE HAVE SPENT MONEY ON IT BEFORE IS AN INDICATION OF ITS EXCELLENCE. AND IF WE GOT SOMETHING THIS EXCELLENT, I THINK WE SHOULD STICK WITH IT AND KEEP FIGHTING FOR IT.

DR. JUELSGAARD: IF I COULD JUST RESPOND TO THAT. WE'RE IN PHASE 1. WE HAVE NO IDEA WHETHER THIS IS WORKABLE OR NOT. PHASE 1 IS JUST A START OF THE SAFETY PROCESS. THE FACT THAT WE'VE AWARDED MONEY BEFORE IS NOT REALLY AN INDICATION OF WHETHER THIS IS EXCELLENT OR EFFECTIVE.

DR. STEWARD: I'M SORRY. I DON'T HAVE

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NUMBERS IN FRONT OF ME, BUT OBVIOUSLY DIABETES IS A HUGE UNMET MEDICAL NEED. AND, STEVE, I TOTALLY --

MR. TORRES: $40 BILLION IN CALIFORNIA ALONE.

DR. STEWARD: THANK YOU, SENATOR TORRES.

YOU KNOW, SOMETIMES IT'S JUST HARD, AND IT TAKES A LONG TIME TO SOLVE PROBLEMS THAT SEEMED EASY AT FIRST. AND I JUST HAVE TO SAY THAT I THINK THIS IS ONE OF THOSE. I THINK WE ALL WOULD HAVE EXPECTED IT MAYBE TO GO MORE QUICKLY, BUT THEY'RE MOVING FORWARD AND IT IS A HUGE UNMET MEDICAL NEED. AND I THINK IN THIS CASE WE SHOULDN'T LOOK TO THE PAST AS AN INDICATION OF MONEY MISSPENT OR ANYTHING LIKE THAT, WHICH IS, I THINK, SORT OF WHAT THE IMPLICATION IS HERE. I DO APPRECIATE THAT, BUT THIS IS ONE THAT I THINK WE HAVE TO STICK WITH AND SEE THROUGH ONE WAY OR THE OTHER. THANK YOU.

DR. PRIETO: I WANT TO THANK SENATOR TORRES FOR BRINGING UP THESE NUMBERS. I AM ALSO VERY FAMILIAR WITH THOSE NUMBERS. I REMEMBER SHORTLY AFTER PROP 71 PASSED AND BEING NAMED TO THE BOARD READING A NEWSPAPER ARTICLE ABOUT WHAT SOME POTENTIAL LOW-HANGING FRUIT FOR STEM CELL THERAPY WOULD BE, AND DIABETES WAS LISTED AS ONE OF THOSE. MAKE SOME MORE PANCREATIC BETA CELLS AND, BOOM,

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WE'RE DONE. IT HAS NEVER BEEN EASY. IT IS SOMETHING WITH A HUGE IMPACT. AND THIS HAS BEEN A TEAM THAT HAS A SOLID APPROACH, VERY WELL-EQUIPPED TO ATTACK THIS PROBLEM, AND MEETING THEIR MILESTONES.

IT'S GIVEN ME PAUSE HOW MUCH MONEY WE'VE PUT INTO THIS, BUT I THINK THEY SPENT THAT MONEY WISELY, AND I THINK IT'S WORTH CONTINUING THIS.

SUPERVISOR SHEEHY: DR. MARTIN.

DR. MARTIN: MAY I JUST ASK A TECHNICAL QUESTION? IN THE REVIEW WERE THE REVIEWERS AWARE OF THE SUCCESSES AND FAILURES OF THE PREVIOUS EFFORTS?

SUPERVISOR SHEEHY: YES. I KIND OF GOT THE SENSE THAT THIS WAS THE LAST SWING OF THE BAT. BUT I THINK IT'S KIND OF LIKE DR. STEWARD WAS SAYING. YOU KNOW, IT'S COMPLICATED BECAUSE IT'S BOTH CELLS AND A DEVICE, AND IT'S PLURIPOTENT CELLS WHICH THEN HAVE BEEN BROUGHT A CERTAIN -- I DON'T THINK BROUGHT ALL THE WAY DOWN THE LINE, BUT THEY BROUGHT PRETTY CLOSE TO FULL DIFFERENTIATION, AND THEN THEY'RE PUT INTO A DEVICE AND IMPLANTED. SO THERE HAVE BEEN SOME ISSUES WITH THE DEVICE, BUT THERE'S ALSO BEEN SOME SUCCESSES WITH THE DEVICE.

SO THAT'S KIND OF WHERE WE ARE. I DON'T KNOW IF YOU WANT TO A FURTHER EXPLANATION OF

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PROGRESS, IF PEOPLE WANT THAT. DO WE HAVE A SCIENCE OFFICER PRESENT WHO'S BEEN WORKING ON THE PROJECT? FOR THOSE OF US -- I WOULD MYSELF, HAVING BEEN HERE WHILE WE REVIEWED IT TIME AND TIME AND AGAIN, TO GIVE US A SENSE JUST BECAUSE I THINK THAT WOULD COLOR THE CONVERSATION. BUT IF THERE'S SOMEBODY WHO'S BEEN WORKING ON THIS PROJECT ON THE TEAM BECAUSE THIS IS ONE OF OUR FIRST -- THIS WAS ACTUALLY IN THE ORIGINAL DISEASE TEAM BUCKET. I THINK WE HAVE THREE PROJECTS FROM THE ORIGINAL DISEASE TEAM BUCKET. I THINK SO. THIS ONE, CAPRICOR, AND THEN I THINK WE'RE GOING TO HEAR ABOUT ONE OF THE OTHER PROJECTS. NO, WE'VE GOT FOUR. WE'VE GOT FOUR. SO WE HAVE BROUGHT SOME PROJECTS PRETTY FAR DOWN THE ROAD. I THINK WE'VE GOT FOUR. WE'LL HEAR TWO OF THEM TODAY -- THREE OF THEM TODAY.

DR. OLSON: FIVE.

SUPERVISOR SHEEHY: FIVE FROM THAT ORIGINAL BUCKET. THAT'S NOT BAD CONSIDERING.

DR. SAMBRANO: SO, MR. SHEEHY, LILA COLLINS IS THE OFFICER THAT MANAGES THESE. SHE IS NOT HERE, BUT I CAN TELL YOU, AT LEAST IN TERMS OF THIS PROJECT, WHERE IT IS AND KIND OF HOW IT RELATES TO PREVIOUS ONES IF YOU'D LIKE TO HEAR.

SO THIS PROPOSAL BRINGS OR ADVANCES A

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CLIN1 AWARD THAT WE SUPPORTED IN ORDER TO ADVANCE A SECOND GENERATION DEVICE. SO WHAT THIS DEVICE ALLOWS IS A GREATER LEVEL OF VASCULARIZATION THAT WAS NOT ACHIEVED IN THE ORIGINAL DEVICE AND CELL COMBINATION THAT THEY ATTEMPTED IN THE TRIALS THAT WE FUNDED BEFORE. THEY ARE CONTINUING TO MOVE FORWARD ON THAT IN TERMS OF WORKING OUT ISSUES WITH THE DEVICE MATERIALS. HOWEVER, THE SECOND GENERATION DEVICE ALLOWS A LEVEL OF VASCULARIZATION THAT OVERCOMES A KEY PROBLEM THAT THEY HAD WITH ENGRAFTMENT. THEY WERE NOT ABLE TO GET VASCULARIZATION, AND THERE IS A RESULTING FOREIGN BODY RESPONSE TO THAT DEVICE.

SO BY VIRTUE OF HAVING THE VASCULARIZATION, EVEN THOUGH THE FOREIGN BODY RESPONSE STILL OCCURS, THEY ARE ABLE TO GET SUCCESSFUL ENGRAFTMENT IN ANIMAL MODELS.

SO THEIR GOAL, SO FAR, HAS SHOWN THAT IN MODELS WHERE THEY WERE UNSUCCESSFUL WITH THE ORIGINAL DEVICE, THEY HAVE HAD SUCCESS WITH THIS SECOND GENERATION DEVICE, AND SO THEY'VE DONE THAT IN ANIMAL MODELS. SO THIS PHASE 1 REPRESENTS MOVING INTO THE CLINIC NOW WITH THIS DEVICE TO TEST IT FOR THE FIRST TIME IN HUMANS.

DR. STEWARD: I KNOW THAT THIS IS, OF

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COURSE, MILESTONE BASED. I WONDER IF YOU COULD SAY A WORD ABOUT MILESTONES.

DR. SAMBRANO: SO WHEN WE INITIATE ANY AWARD, OF COURSE, WE NEGOTIATE MILESTONES. IN PARTICULAR, THIS ONE IS AN AWARD THAT'S STRUCTURED UNDER THREE COHORTS. SO THERE WILL BE ACTUALLY A VERY CRITICAL MILESTONE VERY EARLY IN THE AWARD PERIOD WHERE THEY HAVE JUST A HANDFUL OF PATIENTS THAT WILL BE FIRST TO RECEIVE THIS DEVICE. THEY INTEND TO FILE AN IND AMENDMENT THAT ALLOWS THEM TO MOVE FORWARD. THAT FOR US WILL BE A CRITICAL MILESTONE VERY EARLY.

DR. STEWARD: THANK YOU. BY WAY OF JUST COMMENT TO THE PUBLIC AND A REMINDER, THE WAY THIS IS STRUCTURED -- AND, GIL, PLEASE SAY MORE, ANYBODY FROM CIRM SAY MORE -- IS THAT THAT $20 MILLION IS THE TOTAL POSSIBLE, AND THAT THE FUNDING IS MILESTONE BASED. SO DELIVERY OF THE OTHER BOLUSES OF THIS AWARD WOULD COME BASED ON THE ACHIEVEMENT OF THE FIRST MILESTONE WHICH WOULD COME EARLY IN THE TRIAL.

SO WE'RE NOT TALKING NECESSARILY ABOUT THAT ENTIRE AMOUNT OF THE AWARD HAVING GONE TO THIS PROJECT, JUST TO KIND OF GO BACK TO THE FIRST CONVERSATION.

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SUPERVISOR SHEEHY: OTHER DISCUSSION? COMMENTS? QUESTIONS? ANY PUBLIC COMMENT? CAN WE CALL THE ROLL PLEASE.

MS. BONNEVILLE: DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: ABSTAIN.

MS. BONNEVILLE: SHERRY LANSING. DAVE MARTIN.

DR. MARTIN: YES.

MS. BONNEVILLE: LAUREN MILLER. ADRIANA PADILLA.

DR. PADILLA: YES.

MS. BONNEVILLE: JOE PANETTA.

MR. PANETTA: YES.

MS. BONNEVILLE: FRANCISCO PRIETO.

DR. PRIETO: AYE.

MS. BONNEVILLE: ROBERT QUINT.

DR. QUINT: ABSTAIN.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: YES.

MS. BONNEVILLE: JEFF SHEEHY.

SUPERVISOR SHEEHY: YES.

MS. BONNEVILLE: OS STEWARD.

DR. STEWARD: YES.

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MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MS. BONNEVILLE: MOTION CARRIES.
SUPERVISOR SHEEHY: THANK YOU. SO THE NEXT APPLICATION PLEASE.
DR. SAMBRANO: OKAY. THE NEXT APPLICATION IS CLIN2-09688. THIS IS FOR A PHASE 3 CLINICAL TRIAL OF A HUMAN ACELLULAR VESSEL FOR HEMODIALYSIS. SO THIS THERAPY IS A BIOLOGIC. IT'S GENERATED FROM SMOOTH MUSCLE CELLS IN ORDER TO CREATE AN ACELLULAR DEVICE ONCE THE CELLS ARE REMOVED. AND IT IS INTENDED TO BE A CONDUIT FOR VASCULAR ACCESS FOR PATIENTS THAT UNDERGO HEMODIALYSIS.
THEIR GOAL HERE IS TO COMPLETE A PHASE 3 CLINICAL TRIAL THAT COMPARES THIS ACELLULAR VESSEL TO WHAT IS CONSIDERED THE GOLD STANDARD FOR HEMODIALYSIS, WHICH IS THE AV FISTULA. THE MAJOR PROPOSED ACTIVITIES INCLUDE MANUFACTURE AND DISTRIBUTION OF HAV FOR CLINICAL TESTING, ENROLLMENT OF PATIENTS THAT ARE REQUIRING VASCULAR ACCESS FOR HEMODIALYSIS, AND LONGITUDINAL TEST SUBJECT

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FOLLOW-UP DATA COLLECTION AND ANALYSIS. THE FUNDS REQUESTED IS ABOUT 14 MILLION. THE APPLICANT IS PROVIDING CO-FUNDING OF 26.4 MILLION.

SO THE GWG GAVE THIS A SCORE OF 1. THE NUMBER OF VOTES THAT LED TO THE SCORE OF 1 WERE EIGHT MEMBERS SCORED IT IN TIER I, THERE WERE TWO MEMBERS THAT SCORED IT IN TIER II, AND FIVE THAT SCORED IT IN TIER III. OUR CIRM RECOMMENDATION, BASED ON OVERALL PROCESS AND CONDUCT OF THE REVIEW, IS TO FUND IN THE AWARD AMOUNT OF ABOUT 14 MILLION. MR. SHEEHY.

SUPERVISOR SHEEHY: SO DO I HAVE A MOTION ON THIS APPLICATION?

DR. PRIETO: SO MOVED.

SUPERVISOR SHEEHY: GOT A MOTION TO FUND. DO I HAVE A SECOND?

DR. HIGGINS: SECOND.

SUPERVISOR SHEEHY: SECOND FROM DR. HIGGINS. DO WE HAVE DISCUSSION ON THIS?

DR. JUELSGAARD: I ACTUALLY HAVE A SERIES OF QUESTIONS, BUT THE FIRST RELATES TO THE TRIAL SPONSOR, WHICH IS, AS I UNDERSTAND IT, A FOR-PROFIT ENTERPRISE, PROBABLY A COMPANY, WHICH IS LOCATED IN RESEARCH TRIANGLE PARK, NORTH CAROLINA. THAT'S THEIR HEADQUARTERS. SO WHAT'S THE NEXUS TO

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CALIFORNIA FOR THIS PARTICULAR APPLICATION?

DR. SAMBRANO: SO FOR APPLICANTS, WHAT WE ALLOW, IF THEY'RE NOT FROM CALIFORNIA, WHAT WE LIMIT THEM TO IS ACTIVITIES THAT ARE CONDUCTED IN CALIFORNIA. SO THIS IS A MULTISITE TRIAL. AND SO THIS WOULD SUPPORT THE TRIAL IN CALIFORNIA, OR AT LEAST THOSE SITES THAT WOULD BE CONDUCTING THIS IN CALIFORNIA.

DR. JUELSGAARD: SO MY UNDERSTANDING IS THIS SAME ENTERPRISE RECEIVED A GRANT OF SOME $10 MILLION FOR A DIFFERENT PHASE 3 TRIAL IN 2016. IS THAT RIGHT?

DR. SAMBRANO: YES, THAT'S CORRECT.

DR. JUELSGAARD: DO WE HAVE ANY UNDERSTANDING OF WHAT THE RESULTS ARE WITH REGARD TO THAT TRIAL TO DATE?

DR. SAMBRANO: SO WE DON'T, BUT I CAN TELL YOU KIND OF WHAT THE DIFFERENCES BETWEEN THE TWO TRIALS ARE. SO THE TRIAL THAT WE FUNDED AS A PHASE 3 IS THE SAME DEVICE WHERE THEY ARE COMPARING IT TO A SYNTHETIC GRAFT. A SYNTHETIC GRAFT IS USUALLY THE SECOND IN LINE IN TERMS OF WHAT IS PREFERRED FOR HEMODIALYSIS PATIENTS. BUT THERE'S A BROAD POPULATION THAT GET SYNTHETIC GRAFTS, AND SO THEY ARE COMPARING IT TO THAT.

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FOR THIS PARTICULAR TRIAL, THE GOAL IS TO BROADEN THE INDICATION TO THE BROADEST POSSIBLE POPULATION. THAT INCLUDES THE AV FISTULA PATIENTS. SO HERE THEY WANT TO COMPARE WHETHER THIS BIOLOGIC IS MORE EFFECTIVE THAN THE AV FISTULA. SO IT'S DIFFERENT -- IT'S THE SAME PRODUCT. IT'S A DIFFERENT POPULATION OF PATIENTS THAT THEY ARE TESTING IT. SO THESE ARE TWO PARALLEL TRIALS THAT, AT LEAST FROM THE FDA CORRESPONDENCE THAT WAS PROVIDED, IS FELT BOTH WOULD CONTRIBUTE TO THE OVERALL APPROVAL OF THIS PRODUCT.

DR. JUELSGAARD: SO NEXT QUESTION. DO THEY HAVE A COMPLETION DATE IN MIND FOR THE FIRST TRIAL?

DR. SAMBRANO: THEY DO. IT'S A BLINDED TRIAL. SO THE OUTCOMES OF THAT FIRST TRIAL THAT IS CURRENTLY BEING FUNDED BY CIRM WE DON'T HAVE OUTCOMES FOR. THE ONLY OUTCOMES WE HAVE ARE IN TERMS OF SAFETY. SO THE DSMB HAS REVIEWED THAT, AND THERE HAVE BEEN NO ADVERSE EVENTS THAT HAVE BEEN REPORTED WITH, I THINK, UP TO ABOUT A HUNDRED PATIENTS. AND THE COMPLETION DATE IS AUGUST OF NEXT YEAR.

DR. NIKLASON: CAN I OFFER SOME FACTUAL INFORMATION? MY NAME IS LAURA NIKLASON. I'M THE

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FOUNDER OF HEMOCYTE. SO --

SUPERVISOR SHEEHY: I'M SORRY. THE PROCESS IS KIND OF GETTING AWAY FROM US. WE DIDN'T ASK FOR PUBLIC COMMENT OR RESPONSE. I THINK WE JUST DON'T COME UP AND DO LIKE THAT, BUT I'M HAPPY WITH A MORE ORGANIZED PROCESS.

DR. JUELSGAARD: ANYWAY, AS BEST YOU KNOW, DR. SAMBRANO, IT'S AUGUST OF NEXT YEAR. SO ABOUT A YEAR, A LITTLE LESS THAN THAT AWAY WE WOULD KNOW THE RESULTS OF THAT PARTICULAR PHASE 3 CLINICAL TRIAL.

SO SORT OF LAST QUESTION. WOULD THE RESULTS OF THIS FIRST CLINICAL TRIAL, PHASE 3 CLINICAL TRIAL, THE ONE THAT'S AGAINST THE SYNTHETIC MECHANISM OF DELIVERY, WOULD IT INFORM AT ALL THE POTENTIAL FOR SUCCESS OF THE SECOND CLINICAL TRIAL? IN THE BUSINESS WORLD, WE OFTEN USE SOMETHING CALLED PROBABILITY OF TECHNICAL SUCCESS. AND THAT HELPS INFORM YOU ABOUT WHETHER TO MOVE FORWARD OR NOT, AND YOU BASE THAT ON LOOKING BACKWARDS AND SEEING WHAT HAPPENED.

SO THE QUESTION IS IF YOU GOT A NEGATIVE OUTCOME IN THIS FIRST PHASE 3 CLINICAL TRIAL, WOULD THAT INCREASE THE LIKELIHOOD THAT YOU WOULD HAVE A NEGATIVE OUTCOME IN THE SECOND PHASE 3 CLINICAL TRIAL?

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DR. SAMBRANO: THIS IS WHAT CONCERNED SOME OF THE REVIEWERS AND I THINK WHY YOU HAVE A SPLIT IN THE VOTE. I THINK A LOT OF WHAT WORRIED SOME OF THEM IS THAT THEY WOULD LIKE TO SEE EVIDENCE FROM THE FIRST TRIAL IN ORDER TO GAIN CONFIDENCE THAT THIS ONE WILL ALSO WORK.

IT IS -- I CANNOT SPEAK TO WHETHER OR NOT THIS TRIAL WOULD PREVENT, FOR EXAMPLE, THE CONDUCT OF THE AV FISTULA BECAUSE IT IS A DIFFERENT POPULATION THAT THEY ARE LOOKING AT. BUT THE INDICATION, AT LEAST FROM THE FDA, IS THAT THEY BOTH WOULD CONTRIBUTE TO APPROVAL. IF THAT ONE WERE NOT TO SUCCEED, I DON'T KNOW WHETHER OR NOT IT WOULD NEGATIVELY IMPACT ON THEM BEING ABLE TO CONDUCT THE AV FISTULA.

DR. JUELSGAARD: THE QUESTION IS NOT SO MUCH THEY WOULD BE ABLE TO, BUT WHETHER THEY WOULD SPEND THE MONEY TO DO SO. IF YOU GET A NEGATIVE FIRST PHASE 3 TRIAL, THEN DO YOU DECIDE GO FORWARD WITH THE SECOND OR NOT? THAT'S ULTIMATELY THE QUESTION. AND IT HAS TO DO WITH SOME OF THE CONCERNS OF THE REVIEWERS ABOUT OVERLAPPING PHASE 3 CLINICAL TRIALS. IT'S JUST A TIMING ISSUE. ULTIMATELY IF YOU GET A SUCCESS IN YOUR FIRST PHASE 3 TRIAL, YOU'LL MOVE FORWARD WITH YOUR SECOND.

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MY QUESTION IS REALLY HOW CRITICAL IS IT TO DEVELOP THIS SO RAPIDLY. WOULD IT NOT BE BETTER TO KIND OF WAIT AND SEE WHAT THE DATA SHOWS IN THE FIRST TRIAL? AND IF IT'S POSITIVE, THEN THAT GIVES YOU MORE CONFIDENCE THAT THE SECOND TRIAL MIGHT WELL BE POSITIVE AS WELL. AND IF YOU GET A NEGATIVE OUTCOME, IF THE FDA IS GOING TO REQUIRE TWO PHASE 3 CLINICAL TRIALS APPROVAL, WHICH I UNDERSTAND THEY PROBABLY WOULD, THEN GO BACK TO SORT OF THE STARTING BLOCKS DOING THIS ONE AND YET ANOTHER PHASE 3 CLINICAL TRIAL.

DR. SAMBRANO: AS MENTIONED, THAT CONCERN WAS BROUGHT UP. HOWEVER, THERE WERE SEVERAL OTHER REVIEWERS PRESENT THAT FELT THAT IT WAS UNREASONABLE TO EXPECT THEM TO DO IT SEQUENTIALLY, THAT THIS IS ACTUALLY A PROCESS THAT MOST COMPANIES DO, AT LEAST IN PHASE 3 WHEN THEY'RE LOOKING AT A PRODUCT ACROSS MULTIPLE INDICATIONS, TO FOLLOW THEM IN PARALLEL IF IT'S APPROPRIATE. SO THEY FELT IN THIS CASE THAT IT WAS APPROPRIATE.

SUPERVISOR SHEEHY: I DON'T KNOW IF YOU STILL HAVE A QUESTION, DR. PRIETO, THEN DR. STEWARD, AND THEN DR. MARTIN.

DR. PRIETO: NO, I'M FINE.

DR. STEWARD: SO I'M JUST GOING TO

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ANNOUNCE IN ADVANCE THAT I'M INCLINING TOWARD A NO VOTE ON THIS. AND PART OF THE REASON IS THE FACT THAT THE DISTRIBUTION IN SCORES IS THE WAY THAT IT IS. THE FACT THAT THERE ARE VOTES IN TIER II AND TIER III INDICATE THAT MANY OF THE REVIEWERS THOUGHT THAT THIS PROPOSAL COULD BE IMPROVED, AND MAJOR CONCERNS HAVE ALREADY BEEN EXPRESSED IN TERMS OF TIMING OF THIS TRIAL WITH RESPECT TO THE OTHER.

BUT MY, I THINK, GREATER CONCERN OR ISSUE OR WHATEVER YOU WANT TO SAY IS IMPACT HERE. DOES ANYONE HAVE ANY IDEA HOW MANY CALIFORNIANS WOULD BENEFIT FROM THIS?

DR. PRIETO: I DON'T KNOW THOSE EXACT NUMBERS, BUT END STAGE RENAL DISEASE AND RENAL FAILURE ARE VERY SIGNIFICANT AND VERY EXPENSIVE PROBLEMS. I'M ONE OF THE REVIEWERS FOR THIS GRANT AT THE GWG. I WAS MUCH MORE SKEPTICAL INITIALLY. I DID END UP VOTING TO ADVANCE IT. IT IS A VERY EXPENSIVE PROBLEM. IT'S A SIGNIFICANT SOURCE OF MORBIDITY AND MORTALITY. IF THEY ARE SUCCESSFUL, IT DOESN'T CHANGE THE NATURE OF THE DISEASE IN THE WAY THAT SOME OF OUR TREATMENTS COULD. IT WOULD MAKE TREATMENT MORE SUCCESSFUL WITH FEWER COMPLICATIONS. IT WOULD HAVE A SIGNIFICANT IMPACT AND A POTENTIALLY SIGNIFICANT FINANCIAL IMPACT BECAUSE CURRENTLY,

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BECAUSE OF THE DEFICIENCIES IN EITHER AV FISTULAS OR THE EXISTING SYNTHETIC GRAFTS, THERE ARE FREQUENT REHOSPITALIZATIONS, REOPERATIONS, AND MORBIDITY AND MORTALITY ATTACHED TO THAT. SO THIS HAS THE POTENTIAL TO ELIMINATE MUCH OF THAT.

DR. MARTIN: JUST A QUICK QUESTION ABOUT THE CLINICAL TRIALS. THE DURATION OF THE TRIAL AND THE ENDPOINTS, ARE THOSE THE SAME FOR THESE TWO PHASE 3 TRIALS?

DR. SAMBRANO: YES. AND THE DURATION, I BELIEVE, IS APPROXIMATELY THE SAME. IS THAT TRUE, LAURA?

DR. NIKLASON: YES.

DR. SAMBRANO: YES, THE SAME.

DR. MARTIN: HOW LONG?

DR. NIKLASON: IN THIS TRIAL THERE ARE TWO ENDPOINTS. ONE IS AT SIX MONTHS FUNCTIONAL PATENCY FOR DIALYSIS. THE SECOND ONE IS FUNCTIONAL PATENCY AT ONE YEAR. FOR THE PTFT TRIAL, IT WAS FUNCTIONAL PATENCY FOR DIALYSIS AT ONE YEAR.

DR. MARTIN: FINE.

SUPERVISOR SHEEHY: SO WE SHOULD OPEN IT UP MAYBE TO PUBLIC COMMENT BECAUSE I THINK WE'RE STARTING TO ASK QUESTIONS OF THE APPLICANT. MAYBE THAT CAN HELP CLARIFY THINGS FOR SOME FOLKS.

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MR. ROWLETT: THE LAST COMMENT COULD NOT BE HEARD. IF THEY COULD SPEAK INTO THE MIC PLEASE.

DR. NIKLASON: SO IN ORDER TO CLARIFY THE LAST QUESTION, THE LAST TRIAL AGAINST PTFE HAD A ONE-YEAR ENDPOINT LOOKING AT SUITABILITY FOR DIALYSIS AS COMPARED TO PTFE. THAT'S A TRIAL THAT WAS SUPPORTED AND RECOMMENDED BY THE FDA AS OUR FIRST TRIAL.

THE SECOND TRIAL HAS TWO DIFFERENT ENDPOINTS. ONE LOOKS AT FUNCTION FOR DIALYSIS AT SIX MONTHS. AND THE REASON FOR THAT IS BECAUSE A VERY HIGH FRACTION OF FISTULAS FAIL TO BECOME FUNCTIONAL EVER, AND THAT CUT-OFF POINT CLINICALLY IS USUALLY MADE AT SIX MONTHS. SO WE CHOSE A SIX-MONTH TIME POINT TO GET AN APPLES-TO-APPLES COMPARISON BETWEEN HOW MANY PATIENTS WERE DIALYZING ADEQUATELY WITH OUR GRAFT AS COMPARED TO FISTULA. THERE'S ALSO A FOLLOW-ON POINT AT ONE YEAR TO LOOK AT LONGER TERM OUTCOMES.

SUPERVISOR SHEEHY: AND YOU ARE WELCOME TO ADDRESS ANY OF THE OTHER ISSUES THAT WERE BROUGHT UP IF YOU LIKE TO.

DR. NIKLASON: AS FAR AS THE FIRST TRIAL THAT WE DID IN PTFE COMPARING AGAINST PTFE, WE WERE ACTUALLY VERY GRATIFIED BY THE ENROLLMENT AND BY THE

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UPTAKE AND THE ENTHUSIASM BY CLINICIANS. SO JUST AS A COMPARISON, THE LARGEST OTHER CLINICAL TRIAL THAT'S EVER BEEN DONE IN THE DIALYSIS ACCESS BASE WAS ABOUT 140 PATIENTS, AND THAT TRIAL TOOK ABOUT A YEAR AND A HALF OR TWO YEARS TO ENROLL. OUR TRIAL WAS 350 PATIENTS IN 38 SITES, AND WE ENROLLED IN 16 MONTHS. AND THE REASON THAT UPTAKE WAS SO FAST IS BECAUSE SURGEONS ARE VERY EXCITED ABOUT THIS TECHNOLOGY BECAUSE THEY'RE DESPERATE FOR NEW IDEAS BECAUSE NOTHING IMPORTANT HAS COME DOWN THE PIKE FOR DIALYSIS IN THE LAST 20 OR 30 YEARS.

IN ADDITION, AS FAR AS HOW MANY PATIENTS MIGHT BENEFIT IN CALIFORNIA, IF YOU LOOK AT THE DATA FROM 2016, I THINK THERE WERE ABOUT 140,000 PATIENTS THAT SOUGHT DIALYSIS AT DIALYSIS CENTERS THROUGHOUT THE STATE OF CALIFORNIA IN 2016. OF THOSE, PROBABLY 20 OR 30 PERCENT HAVE PTFE AND PROBABLY 40, 50 PERCENT HAVE A FISTULA. SO ADDING THOSE TWO POPULATIONS TOGETHER, WE BELIEVE THAT WE WOULD HAVE THE POTENTIAL TO BENEFIT PROBABLY 120,000 PATIENTS JUST IN THE STATE OF CALIFORNIA.

IF YOU LOOK AT THE AVERAGE COST OF WHAT IT TAKES TO TAKE CARE OF THESE PATIENTS, IT'S ABOUT $90,000 A YEAR. PROBABLY 20 PERCENT OF THAT IS ACCESS FAILURES AND THE REPEATED TRIPS TO THE

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HOSPITAL THAT THESE PATIENTS HAVE TO ENDURE.

SO I THINK THAT THERE'S A HUGE MEDICAL NEED. THERE'S A HUGE POTENTIAL FINANCIAL UPSIDE BECAUSE WE BELIEVE OUR GRAFT LASTS SUBSTANTIALLY LONGER THAN EITHER PTFE OR THE FISTULA, QUITE FRANKLY. I THINK WE'RE GOING TO HAVE A TREMENDOUS MEDICAL IMPACT AND, FRANKLY, A POSITIVE FISCAL IMPACT.

DR. JUELSGAARD: ON THE FIRST PHASE CLINICAL TRIAL, WHICH YOU SAID ENROLLED IN 16 MONTHS, WHEN IS YOUR PROPOSED UNBLINDING DATE TO EVALUATE THE DATA?

DR. NIKLASON: ELEVEN MONTHS FROM NOW, EARLY SEPTEMBER OF 2018.

DR. JUELSGAARD: THANKS.

DR. NIKLASON: BUT WE DID HAVE -- JUST TO CORRECT ANOTHER POINT OF INFORMATION, WE HAVE HAD QUARTERLY DSMB MEETINGS FOR THAT TRIAL, AND THEY MEET EVERY THREE MONTHS, AND THEY ARE UNBLINDED. AND THEY LOOK AT ALL THE DATA, AND THEY'VE GIVEN US AN ENTHUSIASTIC PLEASE PROCEED AT EACH POINT.

SUPERVISOR SHEEHY: SO OTHER QUESTIONS? I ACTUALLY HAD A QUESTION. IS THIS THE ONLY INDICATION FOR THIS PRODUCT?

DR. NIKLASON: ABSOLUTELY NOT. WE HAVE

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ANOTHER PHASE 1 TRIAL THAT WE'VE COMPLETED ENROLLMENT IN PERIPHERAL VASCULAR DISEASE, AND WE HAVE TWO PHASE 1 TRIALS IN PERIPHERAL VASCULAR DISEASE AND IN TRAUMA, TRAUMATIC VASCULAR INJURY, WHICH ARE UNDER WAY AND WHICH ARE ENROLLING IN THE U.S. RIGHT NOW. SO WE ACTUALLY SEE THIS TECHNOLOGY AS REALLY POTENTIALLY CHANGING THE WAY MUCH OF VASCULAR RECONSTRUCTION AND REPLACEMENT IS DONE IN THE U.S.

SUPERVISOR SHEEHY: THANK YOU. OTHER QUESTIONS? SO I THINK -- ANY OTHER PUBLIC COMMENT? SO WE HAVE A MOTION. I BELIEVE THE MOTION IS TO FUND THAT'S BEEN SECONDED. SO COULD WE CALL THE ROLL PLEASE.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: ABSTAIN.

MS. BONNEVILLE: SHERRY LANSING. DAVE MARTIN.

DR. MARTIN: YES.

MS. BONNEVILLE: LAUREN MILLER. ADRIANA PADILLA.

DR. PADILLA: YES.

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MS. BONNEVILLE: JOE PANETTA.
MR. PANETTA: YES.
MS. BONNEVILLE: FRANCISCO PRIETO.
DR. PRIETO: AYE.
MS. BONNEVILLE: ROBERT QUINT.
DR. QUINT: ABSTAIN.
MS. BONNEVILLE: AL ROWLETT.
MR. ROWLETT: YES.
MS. BONNEVILLE: JEFF SHEEHY.
SUPERVISOR SHEEHY: NO.
MS. BONNEVILLE: OS STEWARD.
DR. STEWARD: I'M GOING TO VOTE NO TOO. I'M SORRY.
MS. BONNEVILLE: JONATHAN THOMAS.
CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: ABSTAIN.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MS. BONNEVILLE: MOTION CARRIES.
DR. JUELSGAARD: WHAT WAS THE TALLY?
MR. TOCHER: THE TALLY IS SEVEN AYE VOTES, FOUR ABSTENTIONS, AND TWO NOES.
SUPERVISOR SHEEHY: PRETTY WELL ALIGNED WITH THE WORKING GROUP. SO THE MOTION CARRIES.

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CONGRATULATIONS TO THE APPLICANT. SEEMED LIKE AN INTERESTING PRODUCT, BUT ANYWAY. SO WE HAVE THE NEXT APPLICATION.

DR. SAMBRANO: THE NEXT APPLICATION IS A CLIN1, SO THIS IS IND-ENABLING STUDIES, 10084, FOR A THERAPY FOR SICKLE CELL DISEASE. THE THERAPY IS A GENETICALLY MODIFIED AUTOLOGOUS BLOOD STEM CELL PRODUCT, AND THE INDICATION IS FOR SEVERE SICKLE CELL DISEASE.

THEIR GOAL IS TO COMPLETE IND-ENABLING ACTIVITIES AND FILE AN IND IN ORDER TO TEST THE THERAPY IN A FUTURE CLINICAL TRIAL. THE MAJOR PROPOSED ACTIVITIES INCLUDE GENERATION OF THE VIRAL VECTOR FOR THE GENE CORRECTION, ESTABLISHING REPRODUCIBILITY OF THEIR MANUFACTURING PROCESS, AND THE PREPARATION AND FILING OF AN IND TO BEGIN A PHASE 1-2 TRIAL. THE FUNDS REQUESTED ARE ABOUT 5.2 MILLION. THERE IS NO CO-FUNDING BEING PROVIDED FOR THIS APPLICATION.

THE GWG SCORE IS A 1. THERE WERE 13 VOTES THAT GAVE IT A SCORE OF 1. THERE WAS ONE MEMBER THAT GAVE IT A SCORE OF 2, NONE A SCORE OF 3. THE CIRM TEAM RECOMMENDATION IS TO FUND FOR THE AWARD AMOUNT OF 5.2 MILLION.

SUPERVISOR SHEEHY: DO I HAVE A MOTION ON

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THIS APPLICATION?

MR. ROWLETT: I SO MOVE.

DR. PRIETO: SECOND.

SUPERVISOR SHEEHY: SECONDED BY DR. PRIETO. ANY BOARD DISCUSSION? ANY PUBLIC COMMENT? COULD WE CALL THE ROLL PLEASE.

DR. JUELSGAARD: JUST ONE QUICK QUESTION. SO I KNOW THERE'S ANOTHER CLINICAL TRIAL IN PHASE 1 GOING ON IN SICKLE CELL DISEASE AT UCLA. DO YOU HAVE ANY INFORMATION ON HOW THAT TRIAL IS PROCEEDING? JUST EXPLAIN THE DIFFERENCES OF MECHANISM OF ACTION BETWEEN THIS TRIAL AND THE OTHER TRIAL.

SUPERVISOR SHEEHY: IT'S A DIFFERENT VECTOR, STEVE. I REVIEWED THIS ONE. SO THIS IS A CRISPR APPLICATION. AM I CORRECT? SO IT'S CRISPR-CAS-9. TO ME, THAT'S LIKE THE BIGGEST DIFFERENCE BETWEEN THE TWO TRIALS. IS THIS THE FIRST CRISPR WE'VE MOVED FORWARD?

DR. SAMBRANO: AT THIS STAGE IT IS. SO THIS IS THE MOST ADVANCED IN TERMS OF A CRISPR-CAS9 SYSTEM. SO IT MEANS IT'S A HOMOLOGOUS RECOMBINATION, SO IT'S SUBSTITUTING COMPLETELY FOR THE GENE. THE APPROACH IN THE OTHER TRIAL IS A LENTIVIRAL APPROACH. SO IT'S A LITTLE BIT

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DIFFERENT. SO IT'S NOT A HOMOLOGOUS RECOMBINATION. IT INSERTS A GENE WHEREVER IT MAY LAND. SO THE EXPECTATION IS THAT THE CRISPR-CAS9 IS MORE PRECISE.

DR. JUELSGAARD: THANK YOU.

DR. MARTIN: THERE ARE, I BELIEVE, OTHER NON-CIRM SUPPORTED TRIALS ONGOING IN SICKLE CELL DISEASE, IS THAT NOT TRUE, WITH A DIFFERENT MECHANISM?

DR. SAMBRANO: THERE ARE WITH LENTIVIRAL. I DON'T KNOW THAT THERE ARE ANY THAT HAVE ACTUALLY ADVANCED USING THE CRISPR-CAS9 SYSTEM.

DR. MARTIN: I THOUGHT THERE WAS A ZINC FINGER ENGINEERED.

DR. SAMBRANO: THERE IS A ZINC FINGER.

DR. MARTIN: DO WE KNOW ANYTHING ABOUT THOSE TRIALS AND THE EFFICACY OF THOSE OR SAFETY ISSUES FOR JUST COMPARATIVE PURPOSES?

DR. SAMBRANO: IN TERMS OF THE ONES THAT WE ARE FUNDING, WHICH INCLUDES THE LENTIVIRAL ONE, THOSE ARE PROGRESSING WELL AND ON TIME. BUT BEYOND THAT, I CANNOT SPEAK TO THEM.

DR. MILLAN: LATER ON THIS AFTERNOON, I DON'T KNOW IF IT'S RELEVANT NOW, BUT DR. TALIB FROM OUR THERAPEUTICS TEAM MANAGES OUR PORTFOLIO THAT DEALS WITH GENE-MODIFIED CELL THERAPIES, INCLUDING

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INDICATIONS SUCH AS SICKLE CELL AND HEMOGLOBINOPATHIES. HE WILL BE PRESENTING OUR PORTFOLIO AND CAN RESPOND TO QUESTIONS ABOUT OTHER COMPETING TECHNOLOGIES. IF YOU WOULD LIKE HIM TO COME IN TODAY, IF IT IMPACTS YOUR SCORE OR YOUR RECOMMENDATION FOR THIS AWARD, WE CAN INVITE HIM IN.

DR. MARTIN: IS HE AWARE OF THIS ACTIVITY OR PROPOSAL, AND IS HE SUPPORTIVE?

DR. MILLAN: YES. THE TEAM TRACKS THIS VERY CLOSELY, AND DR. TALIB IS OUR INTERNAL KIND OF EXPERT IN THIS AREA. HE FOLLOWS THE FIELD. HE IS ALSO THE SCIENCE OFFICER ON THE OTHER SICKLE CELL DISEASE UTILIZING LENTIVIRAL-MODIFIED HEMATOPOIETIC STEM PROGENITORS.

SUPERVISOR SHEEHY: OTHER BOARD COMMENT OR QUESTIONS? PUBLIC COMMENT? CAN WE CALL THE ROLL PLEASE.

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

DR. HIGGINS: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: YES.

MS. BONNEVILLE: SHERRY LANSING. DAVE MARTIN.

DR. MARTIN: YES.

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MS. BONNEVILLE: LAUREN MILLER. ADRIANA PADILLA.

DR. PADILLA: YES.

MS. BONNEVILLE: JOE PANETTA.

MR. PANETTA: YES.

MS. BONNEVILLE: FRANCISCO PRIETO.

DR. PRIETO: AYE.

MS. BONNEVILLE: ROBERT QUINT.

DR. QUINT: YES.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: YES.

MS. BONNEVILLE: JEFF SHEEHY.

SUPERVISOR SHEEHY: YES.

MS. BONNEVILLE: OS STEWARD.

DR. STEWARD: YES.

MS. BONNEVILLE: JONATHAN THOMAS.

CHAIRMAN THOMAS: YES.

MS. BONNEVILLE: ART TORRES.

MR. TORRES: AYE.

MS. BONNEVILLE: DIANE WINOKUR.

MS. WINOKUR: YES.

MS. BONNEVILLE: MOTION CARRIES.

SUPERVISOR SHEEHY: SO CAN WE GO TO THE NEXT APPLICATION PLEASE.

DR. SAMBRANO: THE NEXT APPLICATION IS

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CLIN2-10144. THIS IS A PROPOSAL FOR A PHASE 1B CLINICAL TRIAL OF AN ANTIBODY THERAPY FOR AML. THIS IS A MONOCLONAL ANTIBODY THAT TARGETS CANCER STEM CELLS THAT'S GOING TO BE COMBINED WITH ANOTHER DRUG, AZACITIDINE, IN PATIENTS WITH AML.

THEIR GOAL IS TO COMPLETE A PHASE 1B CLINICAL TRIAL TO ASSESS THE SAFETY AND EFFICACY OF THIS THERAPY. THE MAJOR PROPOSED ACTIVITIES INCLUDE THE ASSESSMENT OF SAFETY AND TOLERANCE OF THE ANTIBODY ALONE OR IN COMBINATION WITH AZACITIDINE, TO ASSESS EFFICACY OF THE ANTIBODY ALONE OR IN COMBINATION, AND THE OPTIMAL DOSING REGIMEN. SO THE FUNDS REQUESTED ARE 5 MILLION. THE APPLICANT IS PROVIDING 7.4 MILLION IN CO-FUNDING.

THE GWG SCORE IS A 1. THERE WAS A UNANIMOUS VOTE OF 14 MEMBERS GIVING IT A SCORE OF 1 FOR THIS APPLICATION. AND CIRM RECOMMENDS FUNDING THE AWARD IN THE AMOUNT OF 5 MILLION FOR THIS APPLICANT.

SUPERVISOR SHEEHY: SO DO I HAVE A MOTION ON THIS APPLICATION?

MR. TORRES: SO MOVED.

SUPERVISOR SHEEHY: MOVED BY SENATOR TORRES. DO WE HAVE A SECOND?

DR. STEWARD: SECOND.

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SUPERVISOR SHEEHY: SECONDED BY DR. STEWARD. ANY BOARD COMMENTS OR QUESTIONS? ANY PUBLIC COMMENT? COULD WE CALL THE ROLL.

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

DR. HIGGINS: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: YES.

MS. BONNEVILLE: SHERRY LANSING. DAVE MARTIN.

DR. MARTIN: YES.

MS. BONNEVILLE: LAUREN MILLER. ADRIANA PADILLA.

DR. PADILLA: YES.

MS. BONNEVILLE: FRANCISCO PRIETO.

DR. PRIETO: AYE.

MS. BONNEVILLE: ROBERT QUINT.

DR. QUINT: YES.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: YES.

MS. BONNEVILLE: JEFF SHEEHY.

SUPERVISOR SHEEHY: YES.

MS. BONNEVILLE: OS STEWARD.

DR. STEWARD: YES.

MS. BONNEVILLE: JONATHAN THOMAS.

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CHAIRMAN THOMAS: YES.
MS. BONNEVILLE: ART TORRES.
MR. TORRES: AYE.
MS. BONNEVILLE: DIANE WINOKUR.
MS. WINOKUR: YES.
MS. BONNEVILLE: MOTION CARRIES.
SUPERVISOR SHEEHY: GREAT. THANK YOU. NEXT APPLICATION PLEASE.
DR. SAMBRANO: THE FINAL APPLICATION IS CLIN2-10248. THIS IS A PROPOSAL FOR A PHASE 1 CLINICAL TRIAL OF A CAR T-CELL THERAPY TO TREAT MALIGNANT GLIOMA. SO THIS IS A T-CELL THERAPY. SO THESE ARE T-CELLS THAT WERE ENGINEERED TO TARGET CANCER STEM CELLS. AND, OF COURSE, THE INDICATION IS FOR MALIGNANT GLIOMAS.
THEIR GOAL IS TO COMPLETE A PHASE 1 CLINICAL TRIAL TO ASSESS THE SAFETY AND PRELIMINARY EFFICACY OF THIS THERAPY. THE MAJOR PROPOSED ACTIVITIES INCLUDE MANUFACTURING OF THE PRODUCT, EVALUATING THE ROUTE OF ADMINISTRATION, AND EVALUATE THE SAFETY AND PRELIMINARY SAFETY OF THE PRODUCT AND PRELIMINARY EFFICACY, DEVELOP AND ESTABLISH REAGENTS AND METHODS FOR A FOLLOW-UP PHASE 2. THE FUNDS REQUESTED ARE 12.75 MILLION. THE APPLICANT IS NOT PROVIDING SPECIFIC CO-FUNDING FOR THIS AWARD.

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THE GWG GAVE THIS A SCORE OF 1 WITH A UNANIMOUS VOTE FOR 15 MEMBERS GIVING IT A SCORE OF 1. THE CIRM TEAM AGREES WITH THIS RECOMMENDATION TO FUND IN THE AWARD AMOUNT OF 12.75 MILLION.

SUPERVISOR SHEEHY: DO I HAVE A MOTION ON THIS APPLICATION?

CHAIRMAN THOMAS: SO MOVED.

SUPERVISOR SHEEHY: MOVED BY CHAIRMAN THOMAS. DO I HAVE A SECOND?

DR. PRIETO: SECOND.

SUPERVISOR SHEEHY: SECONDED BY DR. PRIETO. ANY BOARD DISCUSSION?

DR. MARTIN: WHAT IS THE STEM CELL CONNECTION HERE? ARE THESE REALLY STEM CELLS THAT ARE BEING TARGETED, THE IL 13 RECEPTOR?

DR. SAMBRANO: THERE'S A COUPLE OF CONNECTIONS. SO ONE OF THEM IS THE MEMORY STEM CELLS THAT ARE WITHIN THE PRODUCT ITSELF. SO THAT'S ONE ASPECT. AND THE OTHER IS IT FALLS INTO THE CATEGORY WHERE TARGETING A CANCER STEM CELL FALLS WITHIN THE PURVIEW OF WHAT WE FUND. SO IN THIS CASE THEY ARE TARGETING CANCER STEM CELLS IN GLIOMA.

DR. MARTIN: BUT THE PRODUCT IS STEM CELL-DERIVED?

DR. SAMBRANO: IT INCLUDES STEM CELLS

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WITHIN IT.

CHAIRMAN THOMAS: I'D JUST LIKE TO NOTE I THINK THIS IS PARTICULARLY EXCITING BECAUSE, AS WE ALL KNOW, THERE'S BEEN GREAT ADVANCES IN CAR T AND BLOOD CANCERS. AND THIS IS FUNDING ONE TARGETING SOLID TUMOR, WHICH IS A MUCH TOUGHER NUT TO CRACK. SO I THINK IT'S VERY EXCITING THAT CIRM IS INVOLVED HERE.

SUPERVISOR SHEEHY: ANY OTHER BOARD COMMENT?

DR. MALKAS: ACTUALLY THIS WORK HAS GARNERED QUITE A LOT OF EXCITEMENT.

MR. TOCHER: I'M SORRY. YOU'RE NOT A MEMBER OF THE APPLICATION REVIEW SUBCOMMITTEE.

DR. MALKAS: I SAID NOTHING. NOBODY REMEMBER ANYTHING.

SUPERVISOR SHEEHY: I WOULD SAY, THOUGH, THAT DID KIND OF JOG MY MEMORY. THERE IS AN IMPRESSIVE N OF ONE WHERE AN EARLIER ITERATION OF THIS PRODUCT ACTUALLY AT ONE POINT HAD COMPLETELY CLEARED THE TUMORS, WHICH WERE NOT ONLY IN THE BRAIN, BUT THROUGHOUT THE CENTRAL NERVOUS SYSTEM, IF MY MEMORY SERVES ME RIGHT. SO THE POTENTIAL IS HUGE AND REALLY IN A DISEASE WE HAVE NOTHING.

SO OTHER BOARD COMMENT? ANY PUBLIC

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COMMENT? CAN WE CALL THE ROLL.

MS. BONNEVILLE: ANNE-MARIE DULIEGE.

DAVID HIGGINS.

DR. HIGGINS: YES.

MS. BONNEVILLE: STEVE JUELSGAARD.

MR. JUELSGAARD: YES.

MS. BONNEVILLE: SHERRY LANSING. DAVE MARTIN.

DR. MARTIN: YES.

MS. BONNEVILLE: LAUREN MILLER. ADRIANA PADILLA.

DR. PADILLA: YES.

MS. BONNEVILLE: JOE PANETTA.

MR. PANETTA: YES.

MS. BONNEVILLE: FRANCISCO PRIETO.

DR. PRIETO: AYE.

MS. BONNEVILLE: ROBERT QUINT.

DR. QUINT: YES.

MS. BONNEVILLE: AL ROWLETT.

MR. ROWLETT: YES.

MS. BONNEVILLE: JEFF SHEEHY.

SUPERVISOR SHEEHY: YES.

MS. BONNEVILLE: OS STEWARD.

DR. STEWARD: YES.

MS. BONNEVILLE: JONATHAN THOMAS.

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CHAIRMAN THOMAS: YES.

MS. BONNEVILLE: ART TORRES. DIANE WINOKUR.

MS. WINOKUR: YES.

MR. TORRES: ART TORRES, AYE.

MS. BONNEVILLE: MOTION CARRIES.

SUPERVISOR SHEEHY: DO WE HAVE -- ARE WE DONE? SO, CHAIRMAN THOMAS, THAT CONCLUDES THE BUSINESS OF THE APPLICATION REVIEW SUBCOMMITTEE.

CHAIRMAN THOMAS: THANK YOU VERY MUCH, MR. SUPERVISOR.

WE'RE NOW GOING TO BREAK FOR A WORKING LUNCH. FOR MEMBERS OF THE BOARD, THE LUNCH IS OUT THAT BACK DOOR TO THE LEFT AND CONTINUE ALONG INTO THE KITCHEN. AND SINCE WE HAVE A LIMITED AMOUNT, I'VE BEEN ADMONISHED THAT THIS LUNCH IS FOR BOARD MEMBERS. SO THANK YOU, EVERYBODY. WE WILL RESUME BACK HERE IN TEN MINUTES OR SO.

(A RECESS WAS TAKEN.)

CHAIRMAN THOMAS: SO WHILE WE'RE WAITING FOR A MEMBER OR TWO OF THE BOARD TO JOIN, WE'RE GOING TO GO A BIT OUT OF ORDER HERE AND GO TO THE -- SORRY. LITTLE LOGISTICAL DIFFICULTY HERE. BEAR WITH US, THOSE ON THE PHONE. OKAY.

SO WE ARE GOING TO FLIP DOWN TO ITEM 14,

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WHICH IS CLINICAL PROGRAM UPDATES WHICH, AS YOU KNOW, WE DO FROM TIME TO TIME TO GIVE EVERYBODY PROGRESS REPORTS ON A NUMBER OF THE GREAT CLINICAL TRIALS THAT WE ARE IN THE PROCESS OF FUNDING. AND I'M GOING TO START WITH DR. KAREN RING WHO'S GOING TO INTRODUCE OTHERS FROM THERE.

DR. RING: HELLO, EVERYONE. I HOPE YOU ARE ENJOYING YOUR LUNCH. SO IT'S MY PLEASURE TO SPEAK TO YOU ALL TODAY AND LAUNCH OUR CLINICAL PORTFOLIO OVERVIEW. AND SO THERE'S BEEN A GROWING INTEREST IN OUR CLINICAL TRIALS LATELY. WITH THE LAUNCH OF OUR NEW STRATEGIC PLAN, WE MADE A GOAL OF FUNDING 50 NEW CLINICAL TRIALS BY 2020. AND BY THE BOARD'S VOTE TODAY, YOU CAN SEE THAT WE'RE WELL ON OUR WAY TO REACHING OUR GOAL.

AND SO ONE OF THE THINGS THAT WE WANTED TO DO ON THE COMMUNICATIONS TEAM WAS TO MAKE IT EASIER FOR THE BOARD AND THE PUBLIC AND SCIENTISTS TO SEE ALL THE TRIALS THAT WE ARE FUNDING AND TO GET THE INFORMATION THAT THEY NEED ABOUT THESE TRIALS. SO ON OUR WEBSITE, THIS IS OUR CLINICAL TRIALS PAGE AS IT EXISTS RIGHT NOW. WE JUST HAVE A SIMPLE TABLE LISTING ALL THE TRIALS. AND SO WHAT WE'VE BEEN ABLE TO DO IS TO DEVELOP A CLINICAL DASHBOARD. THIS IS AN INTERACTIVE TABLE THAT WILL ALLOW YOU TO SORT ALL

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OF OUR TRIALS BASED ON DISEASE INDICATION. WE ALSO HAVE A COOL GRAPHIC THAT MY COLLEAGUE TODD DUBNIKOFF MADE WHERE IT WILL LIST THE TOTAL NUMBER OF TRIALS THAT WE'VE FUNDED. SO, AS YOU CAN SEE, THIS IS ALREADY OUT OF DATE AND THE NUMBER SHOULD NOW BE 40.

WE HAVE A GRAPH SHOWING THE DISEASE AREAS AND THE PERCENTAGE OF TRIALS THAT COVER THESE DISEASE AREAS. AND ALSO YOU CAN DOWNLOAD A BROCHURE THAT LISTS ALL OF OUR CLINICAL TRIALS WITH EXPLANATIONS FOR THE PUBLIC ABOUT WHAT STEM CELL THERAPY OR TREATMENT IS BEING DEVELOPED AND TESTED.

SO AS YOU SCROLL DOWN THIS TABLE, YOU CAN SEE THAT FOR EACH TRIAL WE LIST THE DISEASE INDICATION, THE INVESTIGATOR, THE ORGANIZATION, AND THEN MORE INFORMATION ABOUT THE TRIAL, LIKE THE PHASE, WHETHER IT'S RECRUITING, OR IT'S STILL LAUNCHING, AND THEN THEIR TARGETED ENROLLMENT. AND IF YOU WANT TO LEARN MORE ABOUT A SPECIFIC TRIAL, YOU CAN CLICK ON THIS LEARN MORE BUTTON, AND YOU WILL GET A SCORECARD THAT LISTS INFORMATION ABOUT THE TRIAL AND ALSO THE CLINICALTRIALS.GOV LINK SO YOU CAN LEARN MORE ABOUT THE TRIAL THERE, AND ALSO HIGHLIGHTING THE CIRM AWARDS THAT FUND THIS CURRENT TRIAL.

BELOW THAT YOU WILL LEARN MORE DETAILS

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THAT WE GOT FROM OUR SCIENCE OFFICERS ABOUT THE THERAPY, THE DESIGN OF THE TRIAL, THE GOAL, AND THE STATUS, AND ANY NEWS RELEASES RELATED TO THESE CLINICAL TRIALS.

AND THEN ON THE RIGHT, WE HAVE A RESOURCE FOR PATIENTS WHO ARE VISITING THESE PAGES. IF THEY WANT TO LEARN MORE ABOUT CIRM, WE HAVE A LOT OF RESOURCES FOR THEM. AND WE ALSO HAVE THE ALPHA STEM CELL CLINICS AND A NEW VIDEO THAT WE WROTE ABOUT THE STEM CELL TRIALS AND WHAT YOU NEED TO KNOW IF YOU WANT TO PARTICIPATE IN ONE OF THEM.

SO WE HOPE THAT YOU'RE REALLY EXCITED ABOUT THIS. WE'LL SEND OUT A LINK AT THE END OF THE BOARD MEETING SO YOU CAN HAVE ACCESS TO THIS NEW DASHBOARD. WE THINK IT'S A REALLY GREAT WAY TO HIGHLIGHT ALL THE IMPORTANT WORK THAT YOU'VE DECIDED TO FUND. AND, AS YOU CAN SEE, YOU CAN PLAY AROUND AND FIGURE OUT WHAT TRIALS WE'RE FUNDING IN EACH OF THESE DISEASE AREAS, AND THAT WILL ONLY CONTINUE TO GROW.

SO THANK YOU VERY MUCH. NOW I WOULD LIKE TO INTRODUCE SOHEL TALIB WHO WILL GIVE A MORE DETAILED OVERVIEW OF OUR STEM CELL GENE THERAPY PORTFOLIO.

CHAIRMAN THOMAS: SO I JUST WANT TO --

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IT'S A LITTLE TOUGH, WHEN YOU'RE SORT OF SPREAD AROUND THE ROOM, TO SEE HOW MUCH IS ON THIS DASHBOARD AND HOW COMPREHENSIVE IT IS AND WHAT A GREAT RESOURCE IT IS FOR THOSE TRYING TO SEE WHAT'S GOING ON AND GET UPDATES. I JUST WANTED TO CONGRATULATE DR. RING, CONGRATULATE TODD, EVERYBODY WHO WORKED ON THIS. THIS IS A STATE-OF-THE-ART WAY OF MEASURING WHAT'S GOING ON HERE, WHICH IS TERRIFIC. SO CONGRATULATIONS ON THAT. VERY, VERY HELPFUL.

DR. RING: APPRECIATE IT.

(APPLAUSE.)

DR. TALIB: THANK YOU, DR. RING. SO I THINK WHAT I WILL DO IS GIVE YOU AN UPDATE ON OUR CLINICAL PROGRAMS.

SO, MR. CHAIRMAN, MEMBERS OF THE BOARD, AND THE MEMBERS OF THE PUBLIC, I WOULD LIKE TO PRESENT TO YOU THE CLINICAL UPDATES ON THE STEM CELL GENE THERAPY PROGRAMS. SO THE GOAL OF THE THERAPEUTICS TEAM AT CIRM IS TO HAVE 50 CLINICAL TRIALS BY 2020, AND WE ARE MOVING TOWARDS THIS GOAL. SO AS DR. MILLAN POINTED OUT, WE HAVE CURRENTLY 30 ACTIVE CLINICAL TRIALS, WHICH ARE PHASE 1-2, PHASE 3 CLINICAL TRIALS, AND NINE OF OUR INVESTIGATORS ARE PREPARING TO FILE THEIR IND NEXT 18 MONTHS.

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AND JUST TO REMIND YOU, OUR THERAPEUTIC PORTFOLIO IS PRETTY DIVERSE, AND THESE 30 CLINICAL TRIALS WHICH WE ARE FUNDING, THEY ARE TARGETING NUMBER OF DISEASES IN MANY OF THESE DISEASE INDICATIONS.

SO FOR TODAY'S DISCUSSION, I WOULD LIKE TO FOCUS ON HEMATOLOGY AND INFECTIOUS DISEASES AND PROVIDE -- SO WHAT I WILL DO IS GIVE YOU AN UPDATE ON THE STEM CELL GENE THERAPY CLINICAL TRIALS IN THE AREA OF HEMATOLOGY AND INFECTIOUS DISEASE.

SO THIS SLIDE LISTS ALL THE CLINICAL TRIALS WHICH WE ARE FUNDING CURRENTLY. AND AS DR. RING POINTED OUT, THAT WE HAVE INFORMATION ON EACH OF THESE GRANTS IN OUR CLINICAL DASHBOARD. SO FOR THE SAKE OF TIME, I WILL NOT BE UPDATING ON ALL THE CLINICAL TRIALS, BUT PROVIDE YOU HIGHLIGHTS OF SOME OF THESE CLINICAL TRIALS.

SO ONE THING I SHOULD POINT OUT, WHAT IS COMMON AMONG THESE CLINICAL TRIALS WHICH WE ARE FUNDING IS THAT THESE ARE AUTOLOGOUS THERAPIES. THAT IS, THE PATIENT'S OWN BLOOD-FORMING STEM CELLS ARE REMOVED FROM THE PATIENTS, THEY ARE GENE MODIFIED BY ADDING A GENE OR EDITING A GENE, AND THEN THESE GENE-CORRECTED STEM CELLS ARE GIVEN BACK TO THE PATIENT.

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SO THE FIRST TWO CLINICAL TRIALS WHICH ARE LISTED HERE ARE IN THE AREA OF CANCER. THE FIRST ONE WE JUST APPROVED FOR FUNDING, AND THAT IS THE STUDY WHICH DR. BROWN IS CARRYING OUT FOR USING CAR T-CELL IMMUNOTHERAPY FOR AN AGGRESSIVE BRAIN CANCER.

THE SECOND CLINICAL TRIAL WHICH WE ARE FUNDING IS FROM UCLA, AND THAT IS TONY RIBAS. HE IS TARGETING USING CAR T-CELLS FOR TARGETING STAGE 3, STAGE 4 SOLID CANCER.

SO THESE TWO CLINICAL TRIALS ARE USING CAR T-CELL IMMUNOTHERAPY. AS MR. CHAIRMAN POINTED OUT EARLIER, WE ARE EXCITED THAT WE ARE FUNDING THESE CLINICAL TRIALS WHICH ARE GENERATING A LOT OF INTEREST. AS YOU HEARD, THE FIRST CAR T-CELL IMMUNOTHERAPY WAS JUST APPROVED BY THE FDA FOR BLOOD CANCER.

SINCE THESE CLINICAL TRIALS ARE JUST STARTING, I WILL NOT BE GIVING YOU ANY UPDATE ON THESE CLINICAL TRIALS, AND HOPEFULLY IN THE NEXT UPDATES, WE WILL PROVIDE YOU WHEN WE UPDATE YOU ON THE ONCOLOGY PORTFOLIO.

SO THE NEXT CLINICAL TRIALS WHICH WE ARE FUNDING, AND THAT IS DR. JUDY SHIZURU. SHE IS DEVELOPING A CHEMOTHERAPY-FREE CONDITION REGIMEN. AND I'LL PROVIDE YOU AN UPDATE ON THIS CLINICAL

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TRIAL A BIT LATER ON. AND AFTER THAT, THERE ARE THREE CLINICAL TRIALS WHICH WE ARE FUNDING, AND THESE THREE CLINICAL TRIALS IS RARE GENETIC DISEASES, AND THOSE ARE THE CLINICAL TRIALS WHICH ARE IN PHASE 1 AS WELL AS A REGISTRATION CLINICAL TRIAL, AND I'LL PROVIDE YOU THE UPDATE ON THE PROGRESS OF THESE CLINICAL TRIALS.

THE LAST TWO CLINICAL TRIALS WHICH ARE LISTED HERE ARE FOR ANEMIA, AND THESE ARE SICKLE CELL DISEASE AND ALPHA THALASSEMIA, AND I WILL PROVIDE YOU UPDATE ON THE PROGRESS OF THESE CLINICAL TRIALS.

IN THE AREA OF INFECTIOUS DISEASES, CURRENTLY WE ARE FUNDING THREE CLINICAL TRIALS. AND THE GOAL OF THESE THREE CLINICAL TRIALS IS TO HAVE FUNCTIONAL CURE FOR HIV/AIDS. SO THE FIRST CLINICAL TRIAL WHICH WE ARE FUNDING, THAT IS AT CAL-IMMUNE, WHICH IS A CALIFORNIA BIOTECH, IN WHICH CLINICAL TRIAL HAS PROGRESSED AND NOW HAS COMPLETED ENROLLMENT. AND I WILL PROVIDE YOU CLINICAL UPDATE ON THIS PARTICULAR PROGRAM. OTHER TWO PROGRAMS ARE ACTIVE. AND THE CLINICAL TRIAL, WHICH DR. ZAIA IN COLLABORATION WITH SANGAMO BIOSCIENCES, WHICH IS FOR STEM CELL GENE THERAPY FOR HIV, IS VERY CLOSE TO THE FINISH LINE. AND HOPEFULLY IN THE NEXT ROUND, I

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WILL BE ABLE TO SHARE THE CLINICAL RESULTS OF THESE CLINICAL TRIALS.

SO LET ME ELABORATE ON THE STUDY WHICH DR. JUDY SHIZURU IS CARRYING OUT AT STANFORD, AND THAT IS A CHEMOTHERAPY-FREE CONDITION REGIMEN FOR STEM CELL TRANSPLANTATION. THE SIGNIFICANCE OF THIS STUDY IS BASED ON THE FACT THAT ALLOGENEIC TRANSPLANT, THAT IS, USING THE STEM CELLS FROM A HEALTHY INDIVIDUAL, IS ONLY FORM OF CURATIVE TREATMENT FOR CERTAIN RARE CANCER AND BLOOD DISEASES. UNFORTUNATELY IT COMES WITH A PRICE, AND THAT IS THAT CHEMOTHERAPY AND RADIATION, WHICH IS USED TO CREATE A SPACE IN THE BONE MARROW OF THE PATIENT TO ACCEPT INCOMING DONOR CELLS, CREATES A LOT OF SIDE EFFECTS. BECAUSE OF THIS, THESE CURATIVE TREATMENTS, THAT'S BONE MARROW TRANSPLANT FROM A DONOR, CANNOT BE USED FOR A LARGE NUMBER OF PATIENTS WHICH OTHERWISE COULD BENEFIT FROM THESE CURATIVE TREATMENTS.

SO WHAT DR. SHIZURU IS DOING IS THAT SHE IS USING A NOVEL ANTIBODY BASE THAT'S A MONOCLONAL ANTIBODY-BASED TREATMENT WHICH IS NONTOXIC. AND HOPEFULLY IT WILL ENABLE THE POSSIBILITY OF REMOVING THE RADIATION AND CHEMOTHERAPY AND HAVING A NONTOXIC CONDITION REGIMEN FOR ALLOGENEIC BONE MARROW

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TRANSPLANTATION. THIS CLINICAL TRIAL HAS NOW STARTED ENROLLING THE PATIENTS. I'LL BE UPDATING ON THE CLINICAL RESULTS HOPEFULLY THE NEXT TIME.

THE NEXT CLINICAL TRIAL THAT I WOULD LIKE TO GIVE YOU AN UPDATE ABOUT IS CARRIED OUT BY DR. DON KOHN, AND THIS IS IN COLLABORATION WITH A BIOTECH, ORCHARD BIOTHERAPEUTICS. THERE THE GENETIC DISEASE IS AN INHERITED GENETIC DISEASE WHICH IS DUE TO A MUTATION IN AN ENZYME ADA, THAT IS, ADENOSINE DEAMINASE, WHICH IS A CRUCIAL ENZYME REQUIRED FOR MAKING A FUNCTIONAL IMMUNE SYSTEM CELL. BECAUSE THESE CHILDREN WHICH ARE BORN WITH THIS INHERITED DISEASE CANNOT MAKE FUNCTIONAL IMMUNE SYSTEM, THEY ARE KEPT IN ISOLATION AND SOMETIMES CALLED BUBBLE BABY DISEASE. SO THIS PARTICULAR CLINICAL TRIAL IS NOW PROGRESSING AND ACTUALLY IS ONE OF THE MOST ADVANCED CLINICAL TRIALS THAT WE ARE FUNDING. THIS IS A REGISTRATION TRIAL TO GET PERMISSION FROM THE FDA TO HAVE THIS TREATMENT AVAILABLE TO ALL THE PATIENTS.

SO FAR DR. KOHN HAS ENROLLED AND TREATED MORE THAN 40 PATIENTS IN THIS CLINICAL TRIAL, INCLUDING THE NINE PATIENTS WHICH ARE TREATED THIS YEAR AND IS PROGRESSING TOWARDS THE BLA FILING. THE RESULTS OF THIS CLINICAL TRIAL HAS BEEN REMARKABLE.

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THERE'S HUNDRED PERCENT SURVIVAL OF THE PATIENTS WHO HAVE BEEN TREATED SO FAR, AND THERE IS ALSO EFFICACY AS WELL AS THE SAFETY OF THESE PATIENTS.

THIS IS ONE OF THE PATIENTS WHOM YOU HAVE MET IN LAST BOARD MEETING, EVIE. SHE WAS TREATED IN 2012 WHEN SHE WAS FEW MONTHS OLD. NOW EVIE IS SIX YEARS OLD AND IS THRIVING.

THE NEXT CLINICAL TRIAL THAT I WILL TRY TO GIVE YOU AN UPDATE ABOUT IS ANOTHER IMMUNE DEFICIENCY SYNDROME WHICH IS X-CGD, WHICH X-CGD, THAT IS X-LINKED CHRONIC GRANULOMATOUS DISEASE. IN THIS PARTICULAR DISEASE, THE GENE WHICH IS AFFECTED IS DIFFERENT THAN THE ADA SCID, WHICH I PROVIDED YOU UPDATE PREVIOUSLY. SO HERE THE ENZYME WHICH IS AFFECTED MAKES IT DIFFICULT FOR THE NEUTROPHIL, THAT IS THE TYPE OF WHITE BLOOD CELLS, WHICH ARE RESPONSIBLE FOR FIGHTING BACTERIAL AND FUNGAL INFECTIONS. SO THESE CHILDREN WHICH ARE BORN HAVE VERY LITTLE OR NO IMMUNE SYSTEM. AND ONLY CURATIVE TREATMENT FOR THEM IS A TRANSPLANT.

SO THIS IS A MULTICENTER CLINICAL TRIAL IN WHICH DR. KOHN, ALONG WITH HIS COLLABORATORS FROM NATIONAL INSTITUTE OF HEALTH AND BOSTON CHILDREN'S, ARE TREATING THESE PATIENTS WITH AUTOLOGOUS STEM CELL GENE THERAPY IN WHICH THE GENE IS BEING

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CORRECTED AND GIVEN BACK TO THESE PATIENTS.

DR. KOHN AND HIS TEAM HAS ENROLLED FIVE PATIENTS IN THIS CLINICAL TRIAL, AND ALL THESE FIVE PATIENTS SHOW THE ENGRAFTMENT OF GENE-MODIFIED STEM CELLS AS WELL AS IT SHOWS THE EFFICACY FROM THE POINT OF VIEW THAT THE WHITE BLOOD CELLS IN THESE PATIENTS ARE NOW ABLE TO PRODUCE THE ENZYME AND SHOW THE FUNCTIONAL ACTIVITY. SO THE SAFETY OF THESE PATIENTS HAVE BEEN SHOWN. THERE ARE FIVE PATIENTS WHICH HAVE BEEN TREATED. THE FIRST PATIENT IS NOW ALMOST TWO YEARS OUT OF TRANSPLANTATION AND IS DOING WELL. OTHER THREE PATIENTS ARE AT LEAST ONE YEAR OR LESS.

SO ONE OF THE PATIENTS IS NO. 5, WHICH WAS TREATED LAST MONTH, HAS SOME SAE, THAT IS, SEVERE ADVERSE EVENT, WHICH APPEARS TO BE DUE TO HIS UNDERLYING DISEASE. BUT TO BE VERY CAUTIOUS, DR. KOHN HAS CURRENTLY SUSPENDED THIS CLINICAL TRIAL TO ENROLL THE NEXT PATIENT TILL THE DATA MONITORING BOARD AND SAFETY MONITORING BOARD AND THE FDA HAS A CHANCE TO REVIEW THE RESULTS AND THEN ALLOW THEM TO PROCEED.

AT THE MOMENT DR. KOHN HAS FIVE PATIENTS WHICH ARE IN THE LINE WHICH HAVE CONSENTED AND ARE WILLING TO GET -- WHO ARE INTERESTED IN

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PARTICIPATING IN THIS CLINICAL TRIAL.

SO THIS IS ONE OF THE PATIENTS, THE FIRST PATIENT WHICH WAS TREATED IN THIS CLINICAL TRIAL. AND NOW THIS BRANDON IS TWO YEARS OUT, AND HE'S DOING WELL. AND YOU HAD A CHANCE TO MEET WITH HIM LAST YEAR WHEN HE WAS AT THE ICOC MEETING.

THE NEXT TRIAL WHICH I WOULD LIKE TO GIVE YOU AN UPDATE ABOUT IS A COLLABORATION BETWEEN ST. JUDE'S CHILDREN'S HOSPITAL AND UCSF. THE INVESTIGATOR IN THIS CASE IS BRIAN SORRENTINO FROM ST. JUDE AND MORT COWAN FROM UCSF.

THE DISEASE HERE IS X-CGD, THE X-LINKED CHRONIC GRANULOMATOUS DISEASE, IS ALSO AN IMMUNE DEFICIENCY DISEASE. AGAIN, IN THIS CASE THE GENE WHICH IS AFFECTED IS DIFFERENT. THIS IS R2 RECEPTOR, AND THAT IS BECAUSE OF THE GENE DEFECT IN THESE PATIENTS WHICH ARE BORN, THEY CANNOT PRODUCE FUNCTIONAL DT AND NK CELLS, WHICH ARE THE FIRST LINE -- WHICH ARE THE SOLDIERS OF THE IMMUNE SYSTEM. AND BECAUSE OF THIS IMMUNE DEFICIENCY, IT IS CATASTROPHIC. THESE PATIENTS CANNOT MAKE MORE THAN ONE YEAR OF THEIR LIFE IN SOME CASES.

SO WHAT DR. BRIAN SORRENTINO AND MORT COWAN IS USING PATIENT'S OWN STEM CELLS AND GENE MODIFYING THEM AND GIVING BACK TO THE PATIENT. IN

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THIS CASE THEY HAVE ENROLLED SIX PATIENTS SO FAR. TWO OF THEM HAVE BEEN ENROLLED AT UCSF, AND THESE CHILDREN ARE FROM FOUR TO SIX MONTHS OLD. AND THE EVIDENCE OF THE RESTORATION OF THE IMMUNE FUNCTION HAS BEEN THE SAFETY OF THESE PATIENTS HAS BEEN SHOWN SO FAR.

AND THE TWO PATIENTS WHICH ARE TREATED AT UCSF ACTUALLY HAS GONE BACK HOME, AND THEY ARE NO LONGER REQUIRING ANY ISOLATION. AND THIS IS THE BABY RONNIE WHICH GOT TREATED AT UCSF, AND WE ARE ACTUALLY FORTUNATE TO HAVE THE FATHER OF BABY RONNIE PRESENT HERE, PRIYANK, AND HE WILL BE TALKING TO THE BOARD AFTER MY PRESENTATION. SO YOU WILL RATHER HEAR FROM HIM THAN ME. SO I WILL TRY TO WRAP UP IT VERY, VERY QUICKLY SO THAT YOU WILL HAVE A CHANCE TO MEET THE DAD OF BABY RONNIE.

SO NEXT CLINICAL TRIAL THAT I'D LIKE TO GIVE YOU AN UPDATE ABOUT IS ABOUT SICKLE CELL DISEASE. AND THIS IS A DISEASE WHICH IS DEVASTATING. I DON'T HAVE TO TELL YOU ABOUT THAT. IT AFFECTS HUNDRED THOUSAND INDIVIDUALS IN U.S. AND MILLIONS OF PEOPLE IN WORLDWIDE. AND THERE'S NO CURATIVE TREATMENT FOR THIS DISEASE. IT DISPROPORTIONATELY AFFECTS AFRICAN AMERICAN POPULATION AS WELL AS THE HISPANIC POPULATION. SO

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IT IS A TRUE UNMET MEDICAL NEED.

DR. KOHN IS USING A STEM CELL GENE THERAPY TRIAL IN THIS CASE. THIS IS A GENE ADDITION. SO HE'S ADDING A GENE WHICH INHIBITS THE SICKLING OF THE RED CELLS. SO THIS PARTICULAR TRIAL IS NOW PROGRESSING. IN THIS PARTICULAR CLINICAL TRIAL, DR. KOHN HAS ENROLLED FIRST PATIENT AND TREATED.

NOW, THIS CLINICAL TRIAL IS PROGRESSING SLOWLY, AND THEN WE WOULD LIKE TO MOVE -- THE REASON FOR THAT IS THE TECHNICAL CHALLENGE IN GENE MODIFYING ENOUGH STEM CELLS SO THAT THEY CAN PRODUCE ENOUGH OF THE HEMOGLOBIN IN THE PATIENT. AND THAT IS ALSO RELATED TO THE GENE WHICH IS BEING ENROLLED. THAT'S A HEMOGLOBIN GENE. THE GLOBIN GENE IS QUITE A LARGE GENE TO BE GENE MODIFIED AND THEN PUT IT BACK AND TO PRODUCE ENOUGH QUANTITY. COMPARED TO OTHER GENE THERAPY CLINICAL TRIALS WHICH I MENTIONED EARLIER, CGD OR ADA AND X-SCID, IN THOSE CASES THE GENE IS SMALL AND EASY TO GENE MODIFY THE STEM CELLS AND PRODUCE ENOUGH QUANTITY.

IN CASE OF SICKLE CELL DISEASE, BECAUSE OF THE LARGE SIZE OF THE GENE, IT HAS BEEN CHALLENGING. HOWEVER, I SHOULD TELL YOU THAT WHAT WE HAVE IN THIS CASE IS AN EXAMPLE WHERE OUR CLINICAL ADVISORY PANEL IS ABLE TO HELP TO OVERCOME THE TECHNICAL CHALLENGES

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WHICH OUR TEAM IS FACING. SO IN THIS CASE, FOR ALL OUR CLINICAL TRIALS, WE HAVE A CLINICAL ADVISORY PANEL, AND THIS CLINICAL ADVISORY PANEL HELPS THE TEAM IF THEY HAVE ANY PROBLEM OR CHALLENGES DURING THE CLINICAL TRIAL. SO IN THIS PARTICULAR CASE, THE CLINICAL ADVISORY PANEL HELPED THE TEAM TO WORK ON THE TECHNICAL CHALLENGE, AND NOW THEY'RE ABLE TO PRODUCE THE LENTIVIRAL VECTOR WITH HIGH TRANSVECTION EFFICIENCY SO THAT THEY CAN PRODUCE ENOUGH AMOUNT OF GENE-MODIFIED STEM CELLS TO HAVE EFFICACY. SO THEY ACTUALLY ARE NOW BACK ON THE TRACK, AND HOPEFULLY THAT WILL ACCELERATE THIS PARTICULAR PROGRAM TO GO TO NEXT STAGE.

I THINK THERE WAS DISCUSSION ABOUT THAT WE CURRENTLY HAVE TWO OTHER PROGRAMS WHICH ARE FOR THE SICKLE CELL DISEASE. ONE IS THE ONE WHICH YOU JUST APPROVED. THAT IS A PRECLINICAL STUDY DOING IND-ENABLING STUDIES USING CRISPR-CAS. IN THAT CASE IT IS A GENE CORRECTION USING THE CRISPR APPROACH. WE ALSO HAVE A PARTICULAR STUDY WHICH WE ARE FUNDING, ANOTHER TEAM WHICH IS ALSO USING CRISPR APPROACH TO DO THE GENE CORRECTION FOR SICKLE CELL DISEASE, BUT THOSE PROGRAMS ARE LITTLE BIT BEHIND. THIS PROGRAM IS THE MOST ADVANCED. IT'S AN OPEN CLINICAL TRIAL.

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ALSO THERE WAS A QUESTION ABOUT WHETHER THERE'S ANY OTHER CLINICAL TRIALS WHICH ARE GOING ON IN U.S. AT THE MOMENT THERE ARE THREE CLINICAL TRIALS WHICH ARE PROGRESSING. ONE IS BLUEBIRD BIO WHICH IS A BIOTECH COMPANY IN BOSTON, AND THEY HAVE TREATED FEW PATIENTS. IN THAT CASE ALSO THEY HAD DIFFICULTY. ONE OR TWO PATIENTS HAD GOOD AMOUNT OF RED BLOOD CELLS PRODUCED ON THOSE PATIENTS ON SAFETY AND EFFICACY WHILE THE OTHER PATIENTS HAD SOME DIFFICULTY. SO IT HAS BEEN TECHNICAL CHALLENGE USING HEMOGLOBIN.

THE NEXT CLINICAL TRIAL IS WHAT DR. KOHN IS DOING. THIRD CLINICAL TRIAL WHICH IS PROGRESSING IS CINCINNATI, AND THEY HAVE ENROLLED ONE PATIENT. SO IT'S OPEN NOW, SO THEY HAVEN'T REALLY IN THE PUBLIC SHOWN ANY CLINICAL RESULTS OF THAT CLINICAL TRIAL. SO THOSE ARE THE THREE CLINICAL TRIALS WHICH ARE CURRENTLY PROGRESSING AND MOVING TOWARD THAT. AT THE MOMENT THERE IS NO CRISPR-BASED CLINICAL TRIAL OR ZINC FINGER NUCLEAR-BASED CLINICAL TRIAL FOR SICKLE CELL DISEASE. THERE'S SEVERAL PROGRAMS WHICH ARE IN PRECLINICAL STAGE IN OTHER COMPANIES AND OTHER UNIVERSITIES.

SO THIS PARTICULAR PROGRAM IS MOVING. HOPEFULLY WILL PROVIDE SAFETY AS WELL AS THE

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EFFICACY IN THESE PATIENTS.

THE NEXT CLINICAL TRIAL WHICH WE ARE FUNDING WHICH JUST GOT STARTED. YOU APPROVED IT IN LAST ICOC MEETING AND IT'S JUST GETTING STARTED. THIS IS ABOUT, AGAIN, IT'S ANEMIA. THIS IS A DEADLY BLOOD DISEASE, A GENETIC DISEASE IN WHICH CHILDREN HAVE DIFFICULTY MAKING RED BLOOD CELLS. THIS IS PROBABLY THE MOST INNOVATIVE APPROACH IN OUR CLINICAL PORTFOLIO WHICH WE HAVE. WHAT TIPPI MACKENZIE IS DOING IN THIS CASE IS THAT THESE PATIENTS IN THIS CASE THE DEGENERATIVE DEFECT IS IDENTIFIED VERY EARLY IN PREGNANCY. AND THE FETUS IS UNABLE TO PRODUCE RED BLOOD CELLS BECAUSE OF GENE MUTATION, AND ALMOST ALWAYS LEADS TO A STILL BIRTH. AND THERE IS NO TREATMENT FOR THIS RARE GENETIC DISEASE.

SO WHAT DR. TIPPI MACKENZIE IS DOING IN THIS CASE IS THAT SHE IS TRANSFUSING MOTHER'S BLOOD-FORMING STEM CELLS INTO THE FETUS. SO IT'S AN IN UTERO TRANSPLANTATION, WHICH HAS NEVER BEEN DONE BEFORE IN THIS CLINICAL INDICATION. AND THE SCIENTIFIC RATIONALE FOR THAT IS THAT DURING THIS STAGE OF FETAL DEVELOPMENT, THE FETUS HAS NOT DEVELOPED ITS OWN MATURE IMMUNE SYSTEM. SO IT'S ABLE TO ACCEPT THE DONOR STEM CELL WITHOUT REJECTING

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THEM, WHICH DOES NOT HAPPEN AFTER THE BABY IS BORN. SO THIS IS AN IN UTERO TRANSPLANTATION. THE WHOLE IDEA IS THAT ONCE YOU TRANSPLANT THESE MOTHER'S BLOOD-FORMING STEM CELLS, THEY WILL BE ABLE TO PRODUCE THE RED BLOOD CELLS AND RESCUE THE FETUS IN FULL TERM.

THIS PARTICULAR CLINICAL TRIAL IS JUST GETTING STARTED OUT. ALTHOUGH IT'S BEEN TESTED IN RARE GENETIC DISEASE; BUT IF THIS IN UTERO APPROACH WORKS, THEN THAT WILL BE ADVANCEMENT IN THE AREA OF GENETIC DISEASES AND ALL OTHER DISEASES. EVEN IN SICKLE CELL DISEASE, IT MAY BE POSSIBLE THAT YOU COULD DO IN UTERO TRANSPLANTATION, AND THE BABY CAN BE BORN AND WILL BE A CURE FOR THESE RARE GENETIC DISEASES.

THE LAST TRIAL WHICH I WOULD LIKE TO GIVE YOU AN UPDATE ABOUT IS A STEM CELL GENE THERAPY FOR HIV. AND THE SCIENTIFIC RATIONALE FOR THIS PROTOCOL IS BASED ON THE BERLIN PATIENTS THAT MOST OF YOU ARE FAMILIAR WITH. AND THE BERLIN PATIENT CASE WAS THIS PARTICULAR GENTLEMAN HAD HIV, AND HE RECEIVED A STEM CELL TRANSPLANT.

(SHORT INTERRUPTION.)

DR. TALIB: I THINK I SHOULD WRAP UP. SO BASICALLY IN THIS CASE IT HAS BEEN SHOWN THAT THE

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DONOR TRANSPLANT FROM A DONOR WHICH HAVE CCR5 NEGATIVE, WHICH IS A CORECEPTOR FOR HIV. IF YOU GET A TRANSPLANT IN THIS PATIENT, THE BERLIN PATIENT WAS CURED FROM HIS HIV. AND NOW IT'S 12 YEARS OR MORE OUT, AND HE HAS NO SIGN OF HIV. SO IT IS POSSIBLE TO GET A TRANSPLANT FROM A DONOR; HOWEVER, THAT IS A RARE CASE THAT YOU COULD DO IT. SO WHAT IS DONE IN THIS PARTICULAR CLINICAL TRIAL IS TAKING PATIENT'S OWN BLOOD-FORMING STEM CELLS, GENE MODIFYING THEM TO KNOCK OUT THE CCR5 USING SHRNA AND C46, WHICH IS ANOTHER FUSION INHIBITOR, SO BASICALLY PROVIDING RESISTANCE TO HIV. SO WHEN THESE GENE-MODIFIED STEM CELLS ARE TRANSFERRED BACK TO THE PATIENT, THEY WILL GENERATE IMMUNE SYSTEM WHICH WILL BE RESISTANT TO HIV.

AND THIS CLINICAL TRIAL HAS NOW TREATED 12 PATIENTS IN TWO COHORTS AND HAS JUST COMPLETED THE LAST PATIENT. SO THE ENROLLMENT AND THE TREATMENT OF THE 12 PATIENTS HAS BEEN COMPLETED. THEY'RE ABLE TO COMPLETE THIS CLINICAL TRIAL TO BASICALLY SHOW THE SAFETY AND THE FEASIBILITY OF DOING IT BECAUSE IT'S THE FIRST-IN-HUMAN CLINICAL TRIAL OF THIS TYPE USING PATIENT'S OWN BLOOD-FORMING STEM CELLS.

NOW, SINCE THE CLINICAL TRIAL HAS JUST STOPPED, THE DATA ANALYSIS IS GOING ON. SO I DON'T

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HAVE ANY FURTHER INFORMATION ABOUT THE OUTCOME OF THIS CLINICAL STUDY; BUT HOPEFULLY, ONCE THE CLINICAL DATA ANALYSIS IS COMPLETED, I WILL BE ABLE TO REPORT BACK TO YOU IN THE NEXT UPDATE.

NOW, THIS PARTICULAR COMPANY, THAT'S CAL-IMMUNE, IS NOW ACQUIRED BY CSL BEHRING. THIS IS AN AUSTRALIAN COMPANY, AND THEY ARE COMMITTED TO FINISH THE CIRM-FUNDED CLINICAL TRIAL.

I THINK THAT COMPLETES MY PRESENTATION. AGAIN, THE GOAL OF THE CIRM, THE MISSION OF CIRM IS TO DEVELOP STEM CELL TREATMENTS FOR PATIENTS WITH UNMET MEDICAL NEED. I'LL BE HAPPY TO TAKE ANY QUESTIONS WHICH YOU MIGHT HAVE.

DR. STEWARD: I'M JUST CURIOUS ON THAT LAST TRIAL, WHAT'S ITS OUTLOOK FOR THE FUTURE GIVEN THE TRANSITION AND OWNERSHIP, SO TO SPEAK?

DR. TALIB: I'M AFRAID I CANNOT REALLY COMMENT. ONLY THING WE KNOW THAT CSL BEHRING IS COMMITTED TO FINISH THE CIRM CLINICAL TRIAL PORTION OF IT, AND THE REASON THEY HAVE ACQUIRED THIS COMPANY IS HOPEFULLY TO SEED ASSETS IN THIS PARTICULAR AREA. I'M AFRAID I CANNOT REALLY COMMENT IN TERMS OF WHAT WILL HAPPEN.

WE HAVE TWO OTHER CLINICAL TRIALS WHICH ARE PROCEEDING, AND THEY'RE USING A DIFFERENT

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APPROACH. THE IDEA IS THE SAME, TO HAVE STEM CELLS GENE THERAPY FOR FUNCTIONAL CURE FOR HIV, AND THOSE TWO CLINICAL TRIALS ARE PROCEEDING. THE ONE WHICH IS PARTIALLY FUNDED BY SANGAMO BIOSCIENCES, THE COMPANY IN RICHMOND HERE, AND THEY PROCEEDING BECAUSE THEY HAVE OTHER CLINICAL TRIALS FOR HIV USING PATIENT'S T-CELLS, AND THIS IS THE FIRST TIME THEY ARE USING HEMATOPOIETIC STEM CELLS. HOPEFULLY THEY WILL BE ABLE TO PROCEED AND TAKE THAT TRIAL, AND THAT'S ALMOST COMPLETING, SO THEY WILL BE ABLE TO TAKE IT TO NEXT STAGE.

DR. STEWARD: BUT NO ONE ELSE IS TARGETING THE RECEPTOR IN ANY OF THE APPROACHES; IS THAT RIGHT?

DR. TALIB: SO THERE HAVE BEEN -- CCR5 RECEPTOR HAS BEEN TARGETED IN T-CELLS BY SANGAMO BY USING THE T-CELL. AND THAT HAS GONE INTO PHASE 1 AND PHASE 2 CLINICAL TRIALS. THERE HAVE BEEN SOME OF THE PRECLINICAL STUDIES IN WHICH PEOPLE HAVE USED CCR5 TARGETING, AND THOSE CLINICAL TRIALS, AGAIN, BASICALLY SHOW THAT IT IS POSSIBLE THAT YOU COULD GENERATE A RESTRICTION TO HIV INFECTION, BUT THE CLINICAL TRIALS ARE THE ONLY ONES WHICH WILL SHOW WHETHER THAT CAN REALLY LEAD TO A FUNCTIONAL CURE FOR HIV.

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OTHER APPROACHES PEOPLE ARE USING, AGAIN, USING CRISPR TO KNOCK OUT THE CCR5 DIRECTLY INTO THE STEM CELLS OR IN VIVO. SO THOSE ARE ADVANCEMENTS, BUT THOSE CLINICAL TRIALS IS NOT AT THE CLINICAL STAGE AT THE MOMENT.

DR. LUBIN: THANKS FOR THE PRESENTATION. AS YOU KNOW, I'VE BEEN INTERESTED IN SICKLE CELL ANEMIA. BUT ONE OF THE THINGS THAT'S UNIQUE ABOUT THE STATE OF CALIFORNIA IS THAT WE STARTED NEWBORN SCREENING FOR SICKLE CELL. SO WE KNOW EVERY CHILD WITH SICKLE CELL WHEN THEY'RE BORN. AND THEN THEY GET EVALUATED BY THE GENETIC DISEASE PROGRAM. MANY OF THE KIDS COME TO OUR PLACE IN OAKLAND FOR EVALUATION AND ENROLL THEM IN PROGRAMS.

TRANSPLANTS ARE EASIER TO DO IN YOUNG CHILDREN FOR A VARIETY OF REASONS. GIVEN THE TECHNOLOGIES THAT HAVE BEEN APPROVED OR THOUGHT ABOUT, WHETHER IT'S CRISPR-CAS9 OR WHATEVER THEY ARE, WE HAVE AN IDEAL POSITION IN THE STATE OF CALIFORNIA TO BE THE LEADERS IN THE WORLD TO ADDRESS THIS WORLDWIDE GENETIC DISEASE. AND I JUST WANT THE BOARD TO KNOW, BECAUSE YOU'VE APPROVED A FAIR NUMBER OF SICKLE CELL-RELATED THINGS, THAT THIS IS A STATE THAT REALLY HAS DEVOTED ENORMOUS AMOUNT OF ATTENTION TO THAT DISEASE AND TO ITS TREATMENT. AND I WANT TO

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CONGRATULATE YOU AND JUST ACKNOWLEDGE THAT WITH THE WORK THAT SOHEL JUST PRESENTED.

DR. JUELSGAARD: JUST A QUESTION ABOUT THE KOHN SCID TRIAL. SO IF I UNDERSTAND IT CORRECTLY, ON THE FIRST SLIDE, THEY WERE LOOKING TO ENROLL TEN PATIENTS; IS THAT RIGHT?

DR. TALIB: THAT'S RIGHT. IF YOU'RE ASKING ABOUT ADA SCID TRIALS, YES, THEY ARE PLANNING TO HAVE TEN PATIENTS. AND THEY HAVE DONE NINE PATIENTS SO FAR. SO THEY ARE VERY CLOSE TO COMPLETING IT.

MR. JUELSGAARD: THAT WAS GOING TO BE MY QUESTION. DO YOU HAVE ANY SENSE OF WHEN IT MIGHT BE COMPLETED? WHAT IS THE FOLLOW-UP TIME PERIOD? AND WHEN MIGHT WE KNOW, THEN, WHETHER WE HAVE A POSITIVE REGISTRATION TRIAL OR NOT?

DR. TALIB: PERHAPS -- I'M NOT SURE WHETHER IT'S PUBLIC INFORMATION BECAUSE ORCHARD BIOTHERAPEUTICS IS INVOLVED. SO, COUNSEL, LET ME KNOW WHETHER I CAN --

MR. TOCHER: IF YOU'RE NOT SURE --

DR. MILLAN: WE CAN GET THAT INFORMATION.

DR. TALIB: WE CAN GET THAT INFORMATION. FROM OUR POINT OF VIEW, THAT CLINICAL TRIAL IS ALMOST READY TO FINISH. THEY ARE PLANNING TO FINISH

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IT THIS YEAR; THAT IS, OUR TEN PATIENTS. AND, AGAIN, IT'S PUBLIC INFORMATION IN TERMS OF THEY ARE PLANNING TO FILE A BLA. WHEN WILL THAT HAPPEN? I'M AFRAID I MAY NOT BE ABLE TO REVEAL THAT.

CHAIRMAN THOMAS: OTHER COMMENTS BY MEMBERS OF THE BOARD? THANKS, SOHEL. THAT WAS VERY WELL DONE AND A VERY EXCITING COMPONENT OF A GREAT PORTFOLIO. SO WE ALWAYS APPRECIATE YOU COMING TO PRESENT.

WE ARE TRANSITIONING BACK TO DR. RING TO INTRODUCE OUR PATIENT ADVOCATE SPEAKER, WHO HAS A DAUGHTER WHOSE PICTURE IS UP ON SCREEN HERE. SON WITH CURLS.

DR. RING: IT'S MY PLEASURE TO INTRODUCE PRIOSH PRIYANK, WHO IS THE FATHER OF THIS ADORABLE SON NAMED RONNIE. AND HE'S GOING TO SPEAK TODAY ABOUT HIS FAMILY'S EXPERIENCE IN THE UCSF/ST. JUDE SCID TRIAL. THANK YOU SO MUCH FOR BEING HERE.

MR. PRIYANK: HI. THANK YOU FOR INVITING ME OVER HERE AGAIN AND KEVIN. I'M REALLY EXCITED. I WASN'T SURE THAT IT WOULD BE SO MUCH OVERWHELMING, BUT I'M REALLY HAPPY THAT I AM HERE AND TRYING TO SHARE.

DR. DIXON: COULD I ASK THAT YOU SPEAK UP A LITTLE BIT OR GET CLOSER TO THE MIC?

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MR. PRIYANK: RONNIE WAS BORN ON MARCH 9, 2017. WE WERE AT MERCY FOLSOM HOSPITAL. WE WERE VERY EXCITED. ALL THE PRENATAL SCREENINGS OR WHATEVER SCREENING THAT WE DO BEFORE A CHILD IS BORN CAME VERY, VERY GOOD. EVERYTHING WAS PERFECTLY NORMAL. AND WHEN HE WAS DISCHARGED FROM MERCY FOLSOM, HE WAS ABSOLUTELY NORMAL. EVERYTHING WAS VERY GOOD UNTIL WE RECEIVED A CALL FROM OUR PEDIATRICIAN IN FOLSOM SAYING THAT THE SCID, WHICH WE DON'T KNOW WHAT IT'S ABOUT, HE SAID IT'S COMING OUT TO BE POSITIVE, SO CAN YOU GO BACK TO THE HOSPITAL AND GIVE A SAMPLE OF BLOOD AGAIN.

SO WE STARTED DOING GOOGLE ABOUT THE SCID. INITIALLY WE WROTE IT LIKE S-K-I-D, AND THEN WE REALIZED IT'S ACTUALLY SCID. PEOPLE CALL IT SCID HERE. IT WAS VERY, VERY THREATENING. IT WAS VERY, VERY -- EVERYTHING THAT WIKIPEDIA SAYS, EVERYTHING THAT GOOGLE SAYS IS KIND OF VERY SCARY ABOUT SCID.

WE KNOW THAT HE DIDN'T HAVE THE IMMUNE SYSTEM, SO WE WENT BACK TO THE HOSPITAL. IT CAME OUT AGAIN TO BE POSITIVE. THEN WE RECEIVED A CALL FROM UCSF AND DOCTORS VERY KIND ENOUGH TO RECEIVE THE NEWBORN SCREENING. WE ARE VERY GRATEFUL THAT WE ARE IN THE STATE OF CALIFORNIA. WE LIVE IN CALIFORNIA AND THE NEWBORN SCREENING IS HERE. THAT

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SAVED HIS LIFE.

WE CAME TO UCSF AS SOON AS DOCTOR SAID YOU MIGHT HAVE TO COME AS QUICKLY AS POSSIBLE. SO WE RUSHED BY TWO HOURS AND THEN HE WAS ADMITTED HERE. THEY SAID HE'S AN X-LINKED SCID. IT MEANS HE DOESN'T HAVE T-CELLS. HE WOULDN'T HAVE THE NK CELLS AND B CELLS AS WELL, BUT WE'RE NOT SURE. I THINK THEY ARE STILL DOING THE RESEARCH. HE HAS NK CELLS AND HE HAS B CELLS, BUT DOESN'T HAVE T-CELLS. HE DIDN'T HAVE T-CELLS AT THAT POINT OF TIME.

WE WERE INTRODUCED WITH BONE MARROW TRANSPLANT AS WELL AS THE TALKS OF GENE THERAPY WAS GOING ON, BUT THEY SAID HE BEING X-LINKED SCID FROM INDIA, WE ARE FROM INDIA, SO THEY SAID MAYBE THE GENE THERAPY MIGHT NOT BE AVAILABLE FOR HIM BECAUSE THAT TIME THEY WERE NOT SURE HE WAS X-LINKED SCID. LATER ON AFTER ALL THE HLA MATCHING AND ALL THOSE KINDS OF THING, THEY SAID HE IS X-LINKED, AND WE ARE VERY FORTUNATE THAT HE COULD BE TREATED WITH THE GENE THERAPY AS WELL IF WE ARE OKAY WITH THAT.

WE AGAIN STARTED GOOGLING ABOUT IT. AND THE KIND OF WEB PAGES THAT CAME UP WAS VERY POSITIVE ABOUT THE GENE THERAPY COMPARED TO BONE MARROW TRANSPLANT BECAUSE IN BONE MARROW TRANSPLANT WE WERE HEARING ABOUT THE GRAFT VERSUS HOST SITUATION AND

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LOT OF OTHER PROBLEMS GOING ON. SO WE STARTED DOING RESEARCH ON GENE THERAPY FOR THE X-LINKED SCID, AND IT WAS VERY HOPEFUL. MOST OF THE PEOPLE, THEY WERE TALKING ABOUT ADA-SCID AT UCLA, WHICH IS GOING VERY WELL. THE OTHER KIND OF SCIDS WERE ALSO BEING TREATED WITH GENE THERAPY AND WAS GOING VERY WELL.

DOCTORS WERE VERY KIND ENOUGH TO INTRODUCE US WITH ONE OF THE OTHER PATIENT FROM SACRAMENTO, ONE OF THE OTHER PATIENT FROM OTHER AREAS, AND EVERYTHING WAS GOING WELL. SO WE SIGNED THE CONSENT, AND NOW HE'S A VERY, VERY HEALTHY BABY. HE HAS PERFECTLY GOOD T-CELLS. HE HAS PERFECTLY GOOD IMMUNE SYSTEM NOW. WE ARE STILL WAITING FOR OTHER CELLS TO COME UP, BUT HE IS DOING VERY GOOD. WE WERE DISCHARGED TWO WEEKS AGO BACK TO OUR HOME, AND HE'S DOING VERY WELL. I HAVE FEW PICTURES OF HIM OVER HERE.

SO THIS IS THE DAY WHEN -- THIS IS LIKE BEFORE YESTERDAY, I GUESS, WHEN HE HAD HIS FIRST SOLID MEAL, RICE CEREAL WITH FORMULA MILK. THESE ARE ALL OF HIS PICTURES. THIS IS HIM EATING THE FIRST SOLID FEED. THIS IS THE DAY WHEN WE GOT DISCHARGED FROM THE HOSPITAL. SO WE WERE TOLD TO HAVE HIM IN THE MASK BECAUSE THE NUMBERS WERE STILL VERY GOOD, BUT STILL WE WANTED TO TAKE CARE OF HIM,

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STILL WE WANT TO MAKE HIM LIKE IN KIND OF ISOLATION, BUT HE'S GOOD NOW.

THE GENE THERAPY IS KIND OF WORKING VERY, VERY GOOD, AND WE ARE VERY HOPEFUL ABOUT IT. AS SOON AS WE HEARD THAT THERE WILL BE A THING, LIKE THE STEM CELLS WOULD BE COLLECTED OUT OF HIM, THEN IT WILL BE CORRECTED WITH THE LENTIVIRAL AND THEN WILL BE GIVEN BACK TO HIM, WE WERE KIND OF VERY HOPEFUL WITH THE KIND OF TREATMENT PROCEDURE THAT THEY WERE DESCRIBING. IT WAS KIND OF VERY HOPEFUL, AND WE WERE PERFECTLY SURE THAT WE NEED TO SIGN IT UP.

BONE MARROW TRANSPLANT FROM THE BEGINNING SOUNDED VERY KIND OF SCARY. LIKE, WE KNEW THAT I WAS GOING TO BE THE DONOR. AND WHEN MY STEM CELLS GOES INTO MY SON, IT MIGHT WORK VERY WELL FOR HIM. BUT, YOU KNOW, IT COULD BE OVERWHELMING FOR HIM OR COULD NOT WORK FOR HIM. SO GENE THERAPY SOMEHOW SOUNDED VERY RELIEVING, AND WE KNEW THAT IT WOULD DEFINITELY WORK FOR HIM. IT'S WORKING VERY WELL. AND HIS B CELLS COUNT ARE ALSO COMING UP. HIS T-CELLS ARE VERY, VERY GOOD. LAST WE HEARD FROM THE DOCTORS WERE THE AVERAGE POPULATION IN THE WORLD, THE T-CELLS ARE LIKE 53 PERCENT WORKING; BUT FOR HIM, THE FIRST TEST WHEN THE FUNCTIONAL TEST CAME

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BACK, IT WAS LIKE 59 PERCENT OF THIS T-CELLS WERE WORKING. SO WE WERE LIKE IS IT REALLY WORKING BEYOND HUMAN LEVEL, OR IS IT LIKE -- THEY SAID IT'S REALLY, REALLY GOOD. IT'S VERY GOOD NEWS.

HERE ARE SOME OF HIS PICTURES. THIS IS THE PICTURE MY WIFE DESIGNED ON THE DAY WHEN WE RECEIVED THE FIRST T-CELLS. AND THE FIRST T-CELLS THAT WE RECEIVED, I THINK THE COUNT WERE LIKE 129, WHICH IS REALLY, REALLY GOOD. AND THESE ARE THE T-CELLS. MY WIFE JUST DESIGNED IT SAYING DID THE T-CELLS COME? YAY, I CAN SEE SOME NUMBERS. BUT, YEAH, HE'S DOING VERY GOOD. THANK YOU FOR THE TEAM HERE --

(APPLAUSE.)

MR. PRIYANK: -- WHO HAS DONE EVERYTHING. THANK YOU.

DR. DIXON: NICE STORY. WHAT A REALLY NICE STORY.

CHAIRMAN THOMAS: THANK YOU VERY MUCH. COMMENTS FROM MEMBERS OF THE BOARD? I THINK THAT STORY SPOKE FOR ITSELF. THAT'S FANTASTIC. CONGRATULATIONS.

MR. PRIYANK: THANK YOU. THANK YOU VERY MUCH.

DR. LUBIN: SO THAT WAS A BEAUTIFUL STORY.

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JUST LIKE SICKLE CELL, NOW WE SCREEN ALL NEWBORNS FOR IMMUNE DEFICIENCY AT THE TIME THEY'RE BORN IN THE STATE OF CALIFORNIA. AND THE DISCOVERY OF THAT TECHNOLOGY WAS AT UCSF. AND I THINK CIRM MIGHT HAVE BEEN PART OF HOW THAT GOT STARTED. BUT THIS IS GOING TO BE A MORE COMMON THING AS WE'LL SEE MORE KIDS THAT ARE AFFECTED THAN WE DID BEFORE BECAUSE THEY'RE PICKED UP AS PART OF NEWBORN SCREENING.

CHAIRMAN THOMAS: THANK YOU. THANKS VERY MUCH.

MR. PRIYANK: THANK YOU, EVERYONE.

CHAIRMAN THOMAS: OKAY. GOING TO JUMP BACK TO ITEM NO. 9, CONSIDERATION OF APPOINTMENT OF NEW SCIENTIFIC MEMBERS TO THE GRANTS WORKING GROUP. DR. SAMBRANO.

DR. SAMBRANO: THIS SHOULD BE A QUICK ITEM. THERE'S NO SLIDES FOR THIS. THIS IS REGARDING APPOINTMENT OF NEW MEMBERS TO THE GRANTS WORKING GROUP. SO WE ARE PROPOSING THREE NEW MEMBERS. THEY ARE DRS. KRISHANU SAHA, CHRISTOPHER SCULL, AND KHALID SHAH. SO THEIR BIOS WERE PROVIDED TO YOU IN THE MATERIALS. SO IF YOU HAVE QUESTIONS, HAPPY TO ADDRESS THEM.

CHAIRMAN THOMAS: HEARING NONE, I THINK WE CAN HAVE A VOICE VOTE WITH POLLING MEMBERS ON THE

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PHONE.

DR. STEWARD: MOVE APPROVAL.

CHAIRMAN THOMAS: IT'S BEEN MOVED BY DR. STEWARD.

DR. DEAS: SECOND.

CHAIRMAN THOMAS: SECONDED BY DR. DEAS. ANY COMMENTS FROM MEMBERS OF THE PUBLIC? HEARING NONE, WE'RE GOING TO HAVE A VOICE VOTE AND POLLING THOSE ON THE PHONE. ALL THOSE IN FAVOR PLEASE SAY AYE. OPPOSED? ABSTENTIONS? MARIA, PLEASE CALL THE ROLL.

MS. BONNEVILLE: JACK DIXON.

DR. DIXON: AFFIRMATIVE.

MS. BONNEVILLE: JUDY GASSON.

DR. GASSON: AYE.

MS. BONNEVILLE: SHLOMO MELMED.

DR. MELMED: AYE.

MS. BONNEVILLE: JOE PANETTA.

MR. PANETTA: YES.

MS. BONNEVILLE: MOTION CARRIES.

CHAIRMAN THOMAS: THANK YOU. ON TO ITEM 10, CONSIDERATION OF FINAL ADOPTION OF NEW IP RULES FOR NEW AWARDS. MR. JUELSGAARD.

DR. JUELSGAARD: SO GOING BACK TO THE BEGINNING OF THE YEAR, MR. TOCHER IS GOING TO TALK

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TO US, MADE A PROPOSAL FOR CHANGING OUR IP REGULATIONS IN THE SPIRIT OF CIRM 2.0, TRYING TO MAKE THEM A LITTLE MORE COHERENT AND EFFICIENT IN THEIR OPERATION. AND SO WITH THAT, WE APPROVED PUTTING FORTH THESE PROPOSED CHANGES TO THE INTELLECTUAL PROPERTY POLICY FOR PUBLIC COMMENT. AND WE'VE NOW HAD THREE ROUNDS OF COMMENTS. AND WITH THAT, I THINK WE'RE READY TO MOVE FORWARD WITH THE PROPOSAL TO AMEND OUR CURRENT IP POLICY. SO I'M GOING TO ASK SCOTT TO REVIEW THE PROPOSED CHANGES WITH US AND ALSO THE PATH THAT WE TOOK TO GET HERE.

MR. TOCHER: GREAT. THANK YOU, STEVE. SO I WILL JUST REMIND THE BOARD OF THE GOALS OF THE PROJECT. AS STEVE HAS SAID, AS THE CIRM TEAM HAS DONE WITH OUR OTHER POLICIES AND RULES PURSUANT TO 2.0, WE WANT TO ENSURE THAT THE REVENUE SHARING COMPONENTS OF OUR IP REGULATIONS ARE AS CLEAR AND SELF-EXECUTING AS POSSIBLE.

SO BASICALLY AT THE END OF THE DAY, THE TEST WAS THAT INTERPRETING THE RULES SHOULDN'T DEPEND ON TO WHOM YOU'RE SPEAKING AT CIRM OR TO OUR SCIENCE OFFICERS. SO PART OF MAKING THAT POSSIBLE IS ENSURING THAT THE RULES USE OBJECTIVE INSTEAD OF SUBJECTIVE STANDARDS WHERE POSSIBLE. AND THAT MEANS THAT WE SHOULD BE EXPLICIT ABOUT STATING EXPECTED

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OUTCOMES AS OPPOSED TO TRYING TO REQUIRE A TYPE OF BEHAVIOR, SUCH AS USING REASONABLE EFFORTS TO LICENSE, FOR INSTANCE.

AS WE'VE HEARD PRETTY UNIFORMLY SINCE GOING BACK AS EARLY AS 2005 WHEN CIRM FIRST STARTED ITS OUTREACH TO COMPILE ITS FIRST IP POLICY, WE HEARD CONSISTENTLY AND SINCE FROM INDUSTRY THAT THEY ARE ACTUALLY LESS CONCERNED ABOUT A GIVEN BALANCE POINT OR ROYALTY RATE IN OUR REGULATIONS THAN THEY ARE ABOUT THE PREDICTABILITY OF MAKING THAT CALCULATION IN ADVANCE. SO WE WANTED OUR REVENUE SHARING RULES TO BE SIMPLER TO CALCULATE PRIOR TO TAKING AN AWARD AND PROVIDE CERTAINTY AND CONFIDENCE IN THOSE CALCULATIONS.

AND, FINALLY, WE KNEW WE WOULD HAVE A GOOD SYSTEM IN PLACE WHEN THE CIRM TEAM RESOURCES ARE FOCUSED ON CIRM'S STRATEGIC MISSION RATHER THAN EXPENDING OUR EFFORTS GRAPPLING WITH INTERPRETATION OF OUR OWN RULES AND HOW THEY MAY APPLY IN A GIVEN COMPLICATED SITUATION.

SO WITH THOSE CHALLENGES, THE NEW POLICY IS COMPRISED OF A COUPLE OF STRUCTURAL FIXES. AND THAT IS WE HAVE ELIMINATED THE DISPARATE TREATMENT OF AWARDEES, AND WE NOW WILL TREAT ALL AWARDEES ALIKE. AND WE DO THAT BY ELIMINATING THE CONCEPT OF

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LICENSING REVENUE FOR ALL AWARDEES AND FOCUS INSTEAD ON THE COMMERCIAL REVENUE CONCEPT, WHICH IS CURRENTLY APPLICABLE ONLY TO OUR FOR-PROFIT AWARDEES.

NOW, I WANT TO REMIND THE BOARD THAT IN MAKING THESE CHANGES TO OUR REVENUE SHARING RULES, WE HAVE MADE NO CHANGES TO OUR CURRENT ACCESS AND PRICING PROVISIONS AS THOSE ARE PRESERVED. WE BELIEVE THAT BY ELIMINATING THE LICENSING REVENUE CONCEPT AND FOCUSING INSTEAD ON THE NOTION OF COMMERCIAL SUCCESSES, WE BELIEVE WE CAN OPTIMIZE CIRM'S REMAINING RESOURCES WHICH WILL ALLOW US TO FOCUS ON CIRM'S STRATEGIC MISSION. AND THAT BY SIMPLIFYING OUR REVENUE SHARING RULES, WE WILL MAKE THEM EASIER TO UNDERSTAND, EASIER TO EXPLAIN, AND, FINALLY, EASIER TO ADMINISTER.

AS A RESULT, WE BELIEVE THAT POTENTIAL APPLICANTS WILL BE ABLE TO MORE ACCURATELY PREDICT THE COST OF CIRM FUNDING AND, THUS, LIKELY MAKE OUR PROGRAMS ACTUALLY MORE ATTRACTIVE TO FOLLOW-ON INVESTMENT AND COMMERCIALIZATION.

SO AS STEVE JUST MENTIONED, THE PROPOSAL HAS UNDERGONE THE PUBLIC COMMENT PHASE OF THE REGULATORY ADOPTION PROCESS SINCE EARLIER THIS YEAR. AND SUBSTANTIVE INPUT WAS PROVIDED BY STANFORD

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UNIVERSITY AS WELL AS THE UNIVERSITY OF CALIFORNIA OFFICE OF THE PRESIDENT. OVER THE COURSE OF SEVERAL MONTHS AND CORRESPONDENCE AS WELL AS SEVERAL IN-PERSON MEETINGS WITH THESE MEMBERS OF OUR REGULATED COMMUNITY, THE PROPOSAL WAS FURTHER DEVELOPED AND REFINED TO ADDRESS THE CONCERNS THAT THEY RAISE AS WELL AS TO IMPROVE THE POLICY.

I'M HAPPY TO REPORT THAT WE WERE ABLE TO RESOLVE THEIR CONCERNS, AND THE COMMENTERS HAVE INDICATED TO US THAT THEY SUPPORT THE REVISIONS.

SO WITH THE INPUT OF THE REGULATED COMMUNITY AND, OF COURSE, THE GUIDANCE AND EXPERTISE THAT STEVE HAS PROVIDED THE TEAM AS WE DEVELOPED THIS POLICY, IT'S NOW READY FOR FINAL ADOPTION TODAY BY THE BOARD. IF IT IS APPROVED TODAY, WE WILL THEN SHIP THIS OFF TO THE OFFICE OF ADMINISTRATIVE LAW, WHICH WILL CONDUCT ITS STATUTORY MANDATED REVIEW OF THE REGULATIONS, WHICH TYPICALLY TAKES A COUPLE OF MORE MONTHS.

BEN HUANG, WHO IS HERE IN THE AUDIENCE, PLAYED AN INTEGRAL PART IN HELPING TEASE OUT SOLUTIONS TO THE CONCERNS THAT WERE RAISED. SO HE'S HERE. WE'RE HAPPY TO ANSWER ANY QUESTIONS YOU MAY HAVE. BUT IN ANY EVENT, WE'D REQUEST A MOTION ON THE LINES THAT IS HERE ON THE SLIDE ALONG WITH THE

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FINDING THAT I HAVE HERE, WHICH IS A REQUIREMENT OF LEGISLATION THAT ALLOWS THE AGENCY TO MAKE AMENDMENTS TO ITS IP REGULATIONS. SO WITH THAT.

CHAIRMAN THOMAS: THANK YOU, MR. TOCHER. DO I HEAR A MOTION TO THAT EFFECT?

DR. JUELSGAARD: SO MOVED.

CHAIRMAN THOMAS: MOVED BY MR. JUELSGAARD. SECONDED BY --

DR. DULIEGE: SECOND.

CHAIRMAN THOMAS: -- DR. DULIEGE. ANY COMMENTS BY MEMBERS OF THE BOARD? PUBLIC COMMENT?

MR. REED: IT WAS MY UNDERSTANDING THAT THE BUSINESS ABOUT STRIKING PROPOSITION 71 BALANCE THE SHARE OF THE STATE REVENUE WITHOUT HINDERING RESEARCH, I THOUGHT THAT WAS IN THE LANGUAGE OF THE PROPOSITION. AND IF SO, CAN WE CHANGE THAT AND SHOULD WE CHANGE THAT? I THOUGHT THAT WAS A VERY IMPORTANT PIECE OF STRUCTURE. IS THAT SOMETHING THAT CAN BE CHANGED BY THIS MANNER? I THOUGHT THAT WAS IN THE ACTUAL CONSTITUTIONAL AMENDMENT.

CHAIRMAN THOMAS: MR. TOCHER.

MR. TOCHER: WE'RE NOT AMENDING THE LANGUAGE THAT YOU ARE REFERRING TO IN PROP 71 THAT REQUIRES THE AGENCY TO BALANCE THE INTERESTS IN THE STATE'S ABILITY TO BENEFIT FROM THE IP THAT IS

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CREATED WHILE AT THE SAME TIME ENSURING THAT THE RESEARCH IS NOT UNREASONABLY HINDERED. THAT STATUTORY PROVISION WAS UNCHANGED. THERE WAS LEGISLATION BACK IN 2010 THAT CODIFIED THE AGENCY'S REVENUE SHARING PROVISIONS, BUT HAD AN ALLOWANCE THAT THE AGENCY COULD NEVERTHELESS CONTINUE ITS PROCESS OF FINE-TUNING THESE PROVISIONS SO LONG AS IT MADE THE FINDING THAT IS INDICATED HERE.

MR. REED: SO THAT WOULD BE A FINE-TUNING?

MR. TOCHER: THAT'S RIGHT.

CHAIRMAN THOMAS: OTHER PUBLIC COMMENT? ANOTHER VOICE VOTE PLUS ROLL CALL FOR THOSE ON THE PHONE. ALL THOSE IN FAVOR PLEASE SAY AYE. OPPOSED? ABSTENTIONS? MARIA, PLEASE CALL THE ROLL.

MS. BONNEVILLE: JACK DIXON.

DR. DIXON: AYE.

MS. BONNEVILLE: JUDY GASSON.

DR. GASSON: AYE.

MS. BONNEVILLE: SHLOMO MELMED.

DR. MELMED: AYE.

MS. BONNEVILLE: JOE PANETTA.

MR. PANETTA: YES.

MS. BONNEVILLE: MOTION CARRIES.

MR. TOCHER: THANK YOU.

CHAIRMAN THOMAS: THANK YOU, MR. TOCHER.

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AND TO, BEN, THANK YOU AS WELL. APPRECIATE YOUR HARD WORK.

SO ITEM NO. 11 IS DEFERRED. THIS CLIN BUDGET AGENDA ITEM WAS ORIGINALLY PLACED ON THE AGENDA GIVEN THE HIGH VOLUME OF CLIN AWARDS IN THE REVIEW PROCESS AND A RISK THAT THERE WOULD BE MORE AWARDS RECOMMENDED THAN COULD BE COVERED IN THE 2017 ALLOCATION. THIS ITEM WILL NOW BE DEFERRED FOR A COUPLE REASONS. NO. 1, AFTER THE SEPTEMBER GWG REVIEW, THE TEAM HAS DETERMINED THAT THERE IS ENOUGH ALLOTTED IN THE 2017 BUDGET TO COVER GWG RECOMMENDATIONS THROUGH DECEMBER OF 2017.

NO. 2, IN ADDITION, RESEARCH BUDGET SCENARIOS WILL BE DISCUSSED AT THE JOINT TRANSITION/ SCIENCE SUBCOMMITTEE MEETINGS IN NOVEMBER, WHICH I'LL TELL YOU MORE ABOUT IN A MINUTE. INFORMED BY THOSE DISCUSSIONS, THE CIRM TEAM WILL PRESENT A BUDGET PROPOSAL TO THE BOARD IN DECEMBER 2017. SO THAT IS THAT ITEM.

ITEM NO. 12 I'M ALSO GOING TO WORK INTO STATEMENTS THAT I HAVE IN MY CHAIRMAN'S REPORT. SO WE ARE DEFERRING THAT ITEM AS WELL.

BEFORE I GET TO THAT, WE'D LIKE TO HAVE PUBLIC COMMENT -- JEANNE, ARE WE ALL PRESENT HERE -- BY A NUMBER OF PATIENT ADVOCATES AND OTHERS WHO

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WOULD LIKE TO SPEAK TO THE BOARD.

MS. ROBB: HI. NICE TO SEE EVERYBODY. NEW FACES TOO. MY NAME IS JENNIFER ROBB. I'VE HAD PARKINSON'S FOR OVER -- DIAGNOSED FOR OVER 12 YEARS. I BET YOU I'VE HAD IT FOR OVER 25. I REMEMBER MY FIRST HAND FLUTTER. I ALSO REPRESENT SUMMIT4STEMCELL FOUNDATION, AMERICANS FOR CURES, AND THIS GROUP ALSO. I DO APPRECIATE CIRM IMMENSELY. AND CONGRATULATIONS TO DR. MILLAN.

I'D LIKE TO EXPRESS OUR APPRECIATION FOR THE SUPPORT THAT'S BESTOWED UPON THE PARKINSON'S PROJECT AT THE LORING LAB BY CIRM. THANKS TO YOUR SUPPORT, WE ARE THE GLOBAL LEADERS IN THIS PARTICULAR ENDEAVOR REPLACING LOST DOPAMINE NEURONS WITH BRAND NEW DNA-MATCHING DOPAMINE NEURONS.

I'D ALSO LIKE TO EXPRESS OUR GRATITUDE TO THE ICOC AND THE STAFF FOR THEIR TIME AND EFFORTS. I'D LIKE TO SHARE A QUOTE WITH YOU FROM THE COO OF THE LA JOLLA INSTITUTE OF ALLERGY AND IMMUNOLOGY. HIS NAME IS DR. STEVEN WILSON, AND THIS IS IN REGARDS, OF COURSE, TO OUR PROJECT. "PROJECTS SUCH AS THIS TRANSCENDS ALL ORGANIZATIONAL BOUNDARIES AND REQUIRES US TO PULL EVERY LEVER WE CAN IN SUPPORT."

DUE TO THE SUPPORT OF THIS ORGANIZATION, AND THE BRILLIANT GUIDANCE OF DR. LORING AND

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DR. BRAAT-LEAL, THE RESEARCH HAS EXCELLED. AND WITH CONTINUED SUPPORT FROM CIRM, IF OUR FUNDING ALLOWS, WE COULD BEGIN CLINICAL TRIALS IN THE FIRST HALF OF 2019. THAT'S CLOSE. I CANNOT BEGIN TO CONVEY TO YOU THE GROUND SWELL OF ANTICIPATION AND EXCITEMENT WITHIN THE PARKINSON'S COMMUNITY. THE NUMBER OF EMAILS AND PHONE CALLS I RECEIVE GROWS DAILY. ON BEHALF OF ALL THOSE WITH PARKINSON'S, I IMPLORE YOU TO KEEP US ON THE CIRM TRACKS TO SUCCESS WITH YOUR CONTINUED SUPPORT. THANK YOU VERY MUCH.

CHAIRMAN THOMAS: THANK YOU.

MS. MALVY: FIRST OF ALL, CONGRATULATIONS AGAIN, DR. MILLAN. AND THANK YOU SO MUCH FOR CIRM. I'M REALLY HAPPY TO BE BACK AFTER TWO YEARS. I'M HERE AS A PATIENT ADVOCATE FOR MY HUSBAND AND FOR SUMMIT4STEMCELL. MY HUSBAND HAS PARKINSON'S. AND WE ARE SO GRATEFUL TO CIRM FOR GIVING US THIS LEG UP AND GETTING US CLOSER TO THE FINAL PUSH.

TWO YEARS AGO I SPOKE TO ALL OF YOU, AND MY HUSBAND WAS ACTUALLY VERY, VERY ABLE TO SPEAK TO YOU. TODAY HE'S NOT QUITE AS ABLE, SO I'M SPEAKING ON HIS BEHALF. I JUST WANT YOU TO KNOW HE DESCRIBED WHAT'S HAPPENING TO HIM NOW AS A TRAIN, A FREIGHT TRAIN, COMING AT HIM. SO I FEEL LIKE THAT THIS HELP THAT CIRM HAS FUNDED AND THE RESEARCH THAT IS BEING

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DONE BY DR. JEANNE LORING IS OUR ONLY HOPE. AND WE'RE SO GRATEFUL.

TODAY WE ARE SO CLOSE WITH ALL OF THE POSITIVE THINGS THAT HAVE HAPPENED IN THE LAST TWO YEARS WITH THE RESEARCH. IT'S AMAZING. AND THIS WILL HELP SILENCE THE SYMPTOMS AND STRUGGLES OF THE FAMILIES AND PATIENTS THAT ARE CAUSED BY THIS DISEASE. WITH YOUR HELP SO FAR AND IN THE NEAR FUTURE, HOPE AND RESULTS ARE DEFINITELY WHAT KEEP US FIGHTING. AND, AGAIN, I WANT TO THANK YOU ALL. I'M SO GRATEFUL. MARY ROSE MALVY.

MS. GOULD: MY NAME IS SHERRY GOULD. I'M A NURSE PRACTITIONER AT SCRIPPS CLINIC. I'M HERE TO REPRESENT THE CLINICAL CONTINGENT OF OUR EXCITING PROJECT AND JUST BRING IT UP TO EVERYONE'S ATTENTION. TALK ABOUT A DISEASE STATE WITH UNMET NEEDS. I MEAN WITH NEEDS THAT ARE UNMET, RATHER.

SIXTY THOUSAND PEOPLE A YEAR, A YEAR, 60,000 AMERICANS A YEAR ARE DIAGNOSED WITH PARKINSON'S DISEASE. A MILLION OF DIAGNOSED PEOPLE IN THE UNITED STATES HAVE PARKINSON'S AND 10 MILLION WORLDWIDE. IT'S REALLY GOING TO BECOME AN EPIDEMIC. SO JUST SHORT AND SWEET, I JUST WANT TO THANK CIRM. I WANT TO THANK ALL OF YOU FOR YOUR SUPPORT THAT YOU'VE GIVEN US IN THE PAST. AND I REMEMBER RANDY

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MILLS SAID, WELCOME TO THE CIRM FAMILY. AND WE'RE CERTAINLY HOPING THAT WE WILL CONTINUE IN THAT CAPACITY IN THE NEAR FUTURE AS WELL AS THE DISTANT FUTURE. THANK YOU SO MUCH.

DR. LAIKIND: HI. I'M DR. PAUL LAIKIND. I'M PRESIDENT AND CEO OF VIACYTE, THE SAN DIEGO COMPANY WORKING TO DELIVER A FUNCTIONAL CURE TO PATIENTS WITH TYPE 1 DIABETES BASED ON AN EMBRYONIC STEM CELL STARTING POINT.

FIRST, I WANT TO ALSO CONGRATULATE MARIA. WE'RE REALLY EXCITED THAT YOU'VE TAKEN THIS ON. WE KNEW YOU WERE THE RIGHT PERSON FOR THIS, SO IT'S GREAT TO HEAR THAT. BUT I ALSO WANT TO THANK THE ICOC FOR THEIR CONTINUED SUPPORT OF THE IMPORTANT WORK WE'RE DOING. AND I WANT TO THANK YOU ON BEHALF, NOT ONLY OF VIACYTE AND THE TEAM WE HAVE THERE THAT HAVE BEEN WORKING ON THIS PROJECT FOR WELL OVER A DECADE, BUT I REALLY WANT THANK YOU FOR THE MILLIONS OF PATIENTS WITH TYPE 1 DIABETES. AND WE HEAR FROM THESE ON A REGULAR BASIS AND WORK WITH THEM.

CIRM ALONG WITH OUR FRIENDS AT JDRF HAVE BEEN OUR PARTNER ON THIS JOURNEY SINCE THE BEGINNING. AND TOGETHER WE'VE GONE FROM BANKING THE STEM CELLS TO DEVELOPING THE PROCESS FOR PRODUCING

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THE PANCREATIC PROGENITOR CELLS THAT WE ACTUALLY IMPLANT, DEVELOPING THE DEVICES THAT WE USE TO DELIVER THESE CELLS, AND CONDUCTING THE PRECLINICAL WORK, AND NOW TREATING PATIENTS.

AS WAS NOTED DURING THE DISCUSSIONS EARLIER OF THE CLIN2 AWARD, THIS IS NOT OUR FIRST GRANT. HOWEVER, I CAN ASSURE YOU THIS HAS BEEN A VERY RATIONAL PROCESS. AS WAS NOTED, OUR FIRST CLINICAL TRIAL WAS WITH A DEVICE THAT WAS DESIGNED TO PROTECT THE CELLS FROM THE IMMUNE SYSTEM. WE WERE REALLY SWINGING FOR THE FENCES WITH THAT, AND IT WAS THE POTENTIAL TO OPEN IT TO ALL PATIENTS WITH THE DISEASE. HOWEVER, WE KNEW WE WERE SWINGING FOR THE FENCES, AND THIS WAS THE FIRST TIME ANYBODY HAD EVER DONE ANYTHING LIKE THIS IN PATIENTS. THE FIRST TIME AN EMBRYONIC STEM CELL HAD GONE FORWARD, DERIVED PRODUCT GONE FORWARD FOR DIABETES OR FOR MOST ANYTHING, AND CERTAINLY THE FIRST TIME A COMBINATION PRODUCT LIKE THIS WAS INTRODUCED INTO PATIENTS.

SO WE KNEW WE HAD A LOT TO LEARN, BUT PATIENTS ARE THE ONLY PLACE WE CAN LEARN IT. AS MANY OF YOU KNOW, WHEN WE PUT A STEM CELL INTO A PATIENT, IT'S WHAT'S CALLED AN ALLOGENEIC TRANSPLANT. IT'S HUMAN CELL IN A HUMAN, BUT NOT THE

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PATIENT'S CELLS. WHEN WE PUT THAT CELL INTO ANIMALS, IT'S A XENOTRANSPLANT. SO THE ANIMAL MODELS TELL YOU A LOT, BUT THEY DON'T TELL YOU EVERYTHING. WE NEEDED TO GET TO THE PATIENTS. THAT'S WHERE CIRM WORKING WITH US ALLOWED US TO DO THAT, AND WE COLLECTED DATA FROM THOSE FIRST TRIALS. AND THAT DATA HAS BEEN VERY IMPORTANT IN DRIVING THE PROGRAM FORWARD.

BOTH THESE PRODUCTS USE THE EXACT SAME ACTIVE BIOLOGIC, THE STEM CELL-DERIVED PANCREATIC PROGENITOR CELLS THAT ARE EXPECTED TO MATURE TO THE ISLET TISSUES UNDER THE SKIN AFTER IMPLANTATION. THUS, WHAT WE LEARN FROM ONE PRODUCT APPLIES TO THE OTHER. IN FACT, THE IND ON THE FIRST PRODUCT THAT WAS FUNDED BY CIRM IS STILL OPEN. WE'RE STILL WORKING ON THAT. THE PROTOCOL IS STILL OPEN. AND WE ARE WORKING TO IMPROVE THAT DEVICE TO BRING IT BACK INTO THE PATIENTS AND MOVE THAT PRODUCT FORWARD FOR ALL PATIENTS.

IN THE MEANTIME, WE'RE WORKING ON THE NEXT PRODUCT THAT IS TARGETING THE HIGH RISK PATIENTS. NOT ONLY IS THAT HELPING THE SICKEST PATIENTS OUT THERE, THE ONES THAT NEED THE MOST HELP WITH THE GREATEST MEDICAL NEED, BUT IT IS INCREASING OUR UNDERSTANDING OF THIS PROGRAM, THIS AREA, AND WHAT

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WE NEED TO ACCOMPLISH TO TREAT ALL PATIENTS WITH THE DISEASE.

SO WE'RE VERY EXCITED ABOUT THE PROGRESS WE'VE MADE, AND WE COULDN'T HAVE DONE IT WITHOUT THE TECHNICAL AND THE FINANCIAL HELP FROM CIRM. SO ONCE AGAIN, I JUST WANT TO THANK YOU AND LOOK FORWARD TO REPORTING ON OUR CONTINUED PROGRESS.

(APPLAUSE.)

CHAIRMAN THOMAS: THANK YOU.

DR. CHIU: ARLENE CHIU, CITY OF HOPE. TODAY IS A GROUNDBREAKING DAY, AND I JUST WANT TO SAY HOW EXCITED AND PLEASED I AM THAT YOU HAVE CHOSEN A WOMAN TO LEAD THIS GREAT AGENCY. AND I CONGRATULATE YOU.

(APPLAUSE.)

DR. CHIU: THE ONE COMMENT I WANTED TO MAKE WAS I REALLY ENJOYED DR. RING'S NEW DASHBOARD TO SEE THE GREAT PROGRESS THAT THE CLINICAL TRIALS FUNDED BY YOU HAVE MOVED AHEAD. WHAT I WAS HOPING, THAT MAYBE YOU MIGHT CONSIDER PUTTING ON THAT DASHBOARD ALSO OTHER CLINICAL TRIALS THAT HAVE BEEN INITIATED BY GRANTS THAT CIRM PUT FORWARD. THERE ARE MANY PEOPLE WHO, SPURRED BY YOUR FUNDING, HAVE GONE ON TO OTHER PLACES TO GET FUNDING FOR CLINICAL TRIALS. AND THAT'S A TRIBUTE TO YOU THAT YOU GOT

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THEM STARTED. SO THAT WAS ONE THOUGHT THAT CAME TO MY MIND, BUT IT WAS A BEAUTIFUL DASHBOARD. THANK YOU.

CHAIRMAN THOMAS: DR. STEWARD.

DR. STEWARD: I WANT TO TAKE THIS OPPORTUNITY TO GO ON PUBLIC RECORD. ARLENE, YOU TALKED ABOUT BEGINNINGS. AND I WANT TO GO ON RECORD TO POINT OUT TO EVERYBODY WHO DOESN'T KNOW, THERE ARE SOME NEW BOARD MEMBERS WHO MAY NOT, THAT EVERYTHING WE TALK ABOUT TODAY REALLY DERIVES FROM BASIC RESEARCH THAT CIRM FUNDED IN THE VERY BEGINNING. AND SELECTING THAT RESEARCH AND THE RESEARCH THAT'S GONE FORWARD REALLY DEPENDED ON A SCIENTIFIC STRUCTURE, A REVIEW PROCESS, AND AN ENTIRE ORGANIZATION THAT WAS BASED ON GOOD MANAGEMENT. AND I WANT TO CALL OUT DR. ARLENE CHIU AS THE ARCHITECT OF THAT IN THE VERY BEGINNING. AND THANK YOU, ARLENE, FOR GETTING US GOING.

(APPLAUSE.)

CHAIRMAN THOMAS: THANK YOU. ANY OTHER PUBLIC COMMENT ON ANYTHING ANYBODY WOULD CARE TO SPEAK ABOUT? A COMMENT FROM DR. PRIETO.

DR. PRIETO: I'M VERY PROUD ALSO THAT WE'VE CHOSEN A WOMAN TO LEAD AS OUR PRESIDENT AND CEO AND WAS THINKING ABOUT, ALONG THE LINES OF WHAT

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OS SAID, THAT ARLENE WAS PRESENT AT THE CREATION AND GOT MUCH OF THIS ENTERPRISE GOING. I ALSO WANTED TO RECOGNIZE PAT OLSON WHO HAS BEEN WITH US AND LED OUR TEAM.

(APPLAUSE.)

DR. PRIETO: WE HAVE HAD SOME REALLY OUTSTANDING WOMEN IN SCIENCE LEADING US AND WORKING ALONGSIDE US, AND I'M VERY HAPPY TO HAVE BEEN A SMALL PART OF THAT.

CHAIRMAN THOMAS: THANK YOU. ANY OTHER COMMENTS? OKAY. LAST, BUT HOPEFULLY NOT LEAST, I'LL GIVE A FEW COMMENTS IN MY CHAIR'S REPORT.

SO OF CONSIDERABLE INTEREST, AS YOU RECALL, AT THE JUNE BOARD MEETING, I CALLED FOR THE ESTABLISHMENT OF A TRANSITION SEARCH SUBCOMMITTEE TO START THE DIALOGUE GOING ON EXAMINING OPTIONS THAT WE WOULD HAVE TO KEEP CIRM AND ALL OF THE WONDERFUL PROJECTS THAT WE'VE FUNDED GOING BEYOND THE TIME THAT WE USE UP THE FUNDS ALLOTTED TO US FROM PROP 71.

THE IDEA OF THIS MEETING, WHICH TOOK PLACE LAST WEEK, WAS TO PUT FORTH A NUMBER OF OPTIONS IN DIFFERENT CATEGORIES OF WAYS WE COULD CONSIDER SUSTAINING THE AGENCY AND TO, FROM THAT MEETING, HAVE A CHANCE TO DISTILL THAT DOWN AND COME UP WITH

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SORT OF A PROPOSED GAME PLAN ON HOW WE MIGHT PROCEED. THE DIFFERENT CATEGORIES OF THINGS WE TALKED ABOUT, FOR THOSE MEMBERS OF THE BOARD WHO ARE NOT PART OF THE SUBCOMMITTEE AND FOR MEMBERS OF THE PUBLIC, BROKE DOWN INTO THE BALLOT MEASURE CONCEPT, ALTERNATIVE FUND-RAISING CONCEPT, AND A JOINT VENTURE CONCEPT.

WITH RESPECT TO THE BALLOT MEASURES, OF COURSE, TO GET TO A POSITION WHERE WE HAVE A SIMILAR AMOUNT OF FUNDING SUCH AS WE'VE ENJOYED UP TO THIS POINT, BALLOT MEASURE IS REALLY THE BEST WAY TO GO. THERE ARE TWO WAYS TO GET ON THE BALLOT. ONE IS TO QUALIFY THROUGH THE LEGISLATURE. THE OTHER IS TO HAVE A CITIZEN-LED CAMPAIGN SIMILAR TO WHAT BOB KLEIN DID FOR PROP 71 IN 2004.

WE DISCUSSED SOME OF THE PROCEDURES FOR THAT, AND PROS AND CONS OF EACH, AND AGREED, AS WITH THESE OTHER TOPICS, THAT WE WOULD TAKE THESE IDEAS UP IN MORE DETAIL AT A SOON-TO-BE-CALENDARED MEETING, WHICH I'LL DESCRIBE IN A SECOND.

WITH RESPECT TO THE FUND-RAISING, WE HAVE TWO THINGS WE NEED FUNDS FOR. NO. 1 IS ADMINISTRATIVE COSTS IN THE EVENT WE ARE NOT SUCCESSFUL WITH A BALLOT MEASURE OR IN GETTING ALTERNATIVE FUNDS TO COVER IT, WE WOULD NEED TO

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CONTEMPLATE WHAT WE DON'T WANT, WHICH IS A WIND-DOWN, AND A WIND-DOWN WOULD ENTERTAIN AN ADDITIONAL ADMINISTRATIVE COST TO MAKE SURE THAT ALL PROJECTS THAT WE HAVE FUNDED ARE FOLLOWED THROUGH TO THEIR LOGICAL CONCLUSION.

TOWARDS THAT END, I REPORTED THAT WE HAD A COUPLE OF VERY GENEROUS GIFTS THAT CAME TO US FROM BILL BOWES, WHO, AS WE ALL KNOW, VERY SADLY AND TRAGICALLY PASSED AWAY A FEW MONTHS AGO, IN THE AMOUNT OF FIVE MILLION AND ANOTHER GIFT FROM PITCH JOHNSON OF TWO MILLION. THOSE GIFTS, WHICH WERE SECURED BACK, I THINK IT WAS, IN 2015 HAD CONTINGENCIES ATTACHED TO THOSE. GIVEN THE NEED FOR CERTAINTY ON THE FUNDING THAT WE WILL REQUIRE, WE WENT BACK AND TALKED TO PITCH, ON THE ONE HAND, AND TO THE EXECUTOR OF BILL'S ESTATE ON THE OTHER, AND THEY AGREED TO REMOVE THE CONTINGENCIES AND TO ACCELERATE SUCH THAT WE WOULD HAVE FUNDS IN HAND BY NO LATER THAN MARCH 31ST OF 2020.

WITH THAT ADDITIONAL AMOUNT OF FUNDING, IT LOOKS LIKE, AND WE'RE REFINING THIS, THAT WILL CARRY US THROUGH FROM AN ADMINISTRATIVE POINT OF VIEW INTO EARLY 2021.

THE SECOND THING WE TALKED ABOUT WAS FUND-RAISING, AND THERE ARE SORT OF TWO WAYS TO LOOK

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AT THIS. ONE IS THE QUEST FOR LARGE GIFTS FROM PHILANTHROPISTS WHO ARE INTERESTED IN MEDICAL RESEARCH WHO SPECIFICALLY FAVOR REGENERATIVE MEDICINE AND WHO MIGHT ENTERTAIN WHAT I CALLED AN UNRESTRICTED GIFT WHICH COULD BE APPLIED AT CIRM'S DISCRETION ACROSS THE BOARD TO A VARIETY OF INDICATIONS AND DISEASES.

THE SECOND THING WE TALKED ABOUT WAS, AS OPPOSED TO SORT OF LARGE UNRESTRICTED GIFTS, WAS A CO-FUNDING STRATEGY WHERE WE WOULD TALK TO HIGH NET WORTH INDIVIDUALS AND/OR FOUNDATIONS WHO WOULD BE INTERESTED IN JOINING US TO COLLABORATE ON OUR ADVENTURE HERE TO BRING STEM CELL TREATMENTS TO PATIENTS THROUGH CO-FUNDING SELECTED PROJECTS. AND THAT COULD EITHER BE ONE OR TWO OR IT COULD BE MANY. SO THAT WAS A STRATEGY THAT WE TALKED ABOUT.

AND WE DISCUSSED THE JOINT VENTURE MODEL. ONE WAY OF LOOKING AT THAT WOULD BE A PUBLIC PRIVATE PARTNERSHIP, A VARIATION PERHAPS ON THE ATP3 THEME THAT WE DISCUSSED AT GREAT LENGTH HERE THAT WOULD REQUIRE FURTHER REFINEMENT FROM THE WAY WE HAD IT ORIGINALLY STRUCTURED.

A SECOND OPTION WOULD BE IN THE FORM OF EITHER A PARTNERSHIP OR A MERGING WITH SOME VERY LARGE FOUNDATIONS THAT FAVOR MEDICAL RESEARCH. AND

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VERY LARGE MEANING IN SORT OF ORDER, AS I MENTIONED, GATES OR WELLCOME OR CHAN ZUCKERBERG OR WHATEVER, AND THAT COULD TAKE THE FORM OF THEM POTENTIALLY HELPING US TO FUND. OR IF WE GOT DOWN TO A SITUATION WHERE WE WERE WINDING DOWN, WHICH, AGAIN, IS CERTAINLY NOBODY'S GOAL HERE, TO THINK ABOUT HAVING OUR HUMAN CAPITAL AND IP SET UP AND ALL OF OUR DIFFERENT ELEMENTS HERE, GRANTS MANAGEMENT THAT MAKE THIS SUCH A SUCCESSFUL OPERATION, TO MERGE INTO ONE OF THOSE ENTITIES.

SO WE HAD A SORT OF ROBUST DISCUSSION ABOUT THESE THREE DIFFERENT CATEGORIES OF IDEAS; AND THE TAKEAWAY FROM THAT WAS, AGAIN, USING WHAT WE HEARD, HAVING GIVEN ALL MEMBERS OF THE SUBCOMMITTEE OPPORTUNITIES TO TALK ABOUT ALL THESE DIFFERENT THINGS, TO DISTILL DOWN INTO A TARGETED PLAN.

SECOND IDEA WAS WE WANTED TO COME BACK FOR A FURTHER DISCUSSION ON THIS TOPIC, IN THE INTERIM HAVING TIME TO REFLECT ON THE FIRST MEETING AND ADD TO THAT MEETING, A CONSIDERATION OF HOW WE DEPLOY THE FUNDS THAT WE HAVE LEFT SORT OF IRRESPECTIVE OF THE FUND-RAISING PLAN. AS DR. MILLAN LAID OUT, WE HAVE A CERTAIN AMOUNT IN THE BIG BUCKET. HOW ARE WE GOING TO UTILIZE THAT? THERE ARE MANY THINGS THAT ARE PART AND PARCEL OF THAT QUESTION. DO WE WANT TO

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STICK TO THE STRATEGIC PLAN AND THE TIMING OF THAT? DO WE WANT TO POTENTIALLY ACCELERATE GIVING OUT AWARDS? HOW DOES THAT AFFECT LONG-TERM FUND AVAILABILITY? WHAT SORT OF MOVE SHOULD WE TAKE TO STRIKE A BALANCE BETWEEN DOING THE BEST BY PATIENTS THAT WE CAN AND UTILIZING WHAT WE HAVE LEFT TO DO SO?

SO THOSE LATTER QUESTIONS TOUCH ON TOPICS THAT ARE SQUARELY IN THE DOMAIN OF THE SCIENCE SUBCOMMITTEE. SO WHAT I CALLED FOR AT THE END OF THAT TRANSITION SUBCOMMITTEE WAS A MEETING, A JOINT MEETING OF TWO SUBCOMMITTEES, TRANSITION AND SCIENCE, WHICH WE ARE NOW GOING TO HOLD, FOR EVERYBODY'S BENEFIT, ON NOVEMBER 2D FROM ONE TO FOUR. THAT MEETING WILL BE HERE. EVERYBODY IS WELCOME. AND SO THAT IS A SUMMARY OF THAT DISCUSSION, AND I'M HAPPY TO ANSWER ANY QUESTIONS ALTHOUGH I WILL NOTE THAT WE SPECIFICALLY HAVE SAID THAT WE WANTED TO WAIT FOR THAT JOINT MEETING TO CONVENE TO ADDRESS A LOT OF THESE THINGS BECAUSE THEY REQUIRE EVERYBODY'S PARTICIPATION OF THOSE TWO, WHICH MAY OR MAY NOT BE HERE TODAY.

ANY COMMENTS FROM MEMBERS OF THE BOARD? OKAY. THANK YOU. AND THANK YOU, BY THE WAY, ALL THE MEMBERS OF THE TRANSITION SUBCOMMITTEE AND FOR

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EVERYBODY WHO'S INTERESTED IN THIS. THIS IS OBVIOUSLY A VERY FRONT-BURNER ISSUE WITH US, AND WE ARE GOING TO DO EVERYTHING WE CAN TO MAKE A SUSTAINED EFFORT HAPPEN.

I JUST WANTED TO SAY A FEW WORDS ABOUT A COUPLE OF PROGRAMS THAT WE HAVE ANNUALLY THAT ARE SO GOOD THAT I ALWAYS LIKE TO COMMENT ON THEM. THIS GETS TO OUR EDUCATIONAL COMPONENT. FIRST IS THE BRIDGES PROGRAM. AND I'M NOT SURE IF SENATOR TORRES IS ON THE LINE STILL. HE MAY BE IN THE AIR. BUT HE WAS, OF COURSE, INSTRUMENTAL IN THE ESTABLISHMENT OF THIS PROGRAM. AND I'M GOING TO JUST TO GIVE YOU A FEW HIGHLIGHTS FROM THIS YEAR.

THE MEETING TOOK PLACE IN LATE JULY IN SAN DIEGO. WE HAD 124 BRIDGES SCHOLARS IN ATTENDANCE AND JUST ABOUT 200 PEOPLE IN TOTAL. THIS YEAR WE DID THINGS A LITTLE DIFFERENTLY THAN WE HAVE IN THE PAST. NORMALLY WE'VE RUN THE EVENT. THIS YEAR WE HANDED OVER THE PLANNING AND EXECUTION OF THE MEETING DIRECTLY TO THE BRIDGES PROGRAMS THROUGH A CIRM CONFERENCE GRANT AWARD. SAN DIEGO STATE AND THEIR BRIDGES PROGRAM WON THAT GRANT, DID A TERRIFIC JOB ARRANGING EVERYTHING, ALL LOGISTICALLY THAT MADE IT RUN VERY SMOOTHLY AND LIKE CLOCKWORK.

WE'RE CURRENTLY OFFERING A CONFERENCE

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GRANT TO HOST THE 2018 AND 2019 BRIDGES ANNUAL MEETINGS AND EXPECT APPLICATIONS EITHER THIS MONTH OR NEXT MONTH. THIS MONTH. THIS MONTH DOESN'T HAVE A LOT LEFT, SO WE'RE GOING TO BE GETTING THEM IN SOON.

THE STUDENTS THEMSELVES, AS THEY ALWAYS DO EVERY YEAR, HEARD FASCINATING SCIENTIFIC PRESENTATIONS FROM OVER A DOZEN RESEARCHERS IN THE FILED, INCLUDING FOUR BRIDGES ALUMNI, SOME FROM ALL THE WAY BACK TO THE FIRST YEAR OF THE PROGRAM IN 2009. THEY ALSO HEARD A VERY POIGNANT KEYNOTE ADDRESS BY ALICIA PADILLA VACCARO'S, LITTLE EVIE, OUR POSTER GIRL'S MOTHER, WHO IF YOU'VE NOT HEARD HER IS JUST SUCH A WONDERFUL SPEAKER AND SO COMPELLING. AND AS ALWAYS, YOU CAN'T HEAR IT WITHOUT IT MAKING YOU CRY BASICALLY. THIS WAS NO DIFFERENT. SO SHE SPOKE ABOUT EVIE, WHO, AS YOU HEARD EARLIER FROM SOHEL, WAS DIAGNOSED WITH SCID FIRST FEW MONTHS OF LIFE. AND AS YOU HEARD, THAT'S A DISEASE THAT IF YOU CONTRACT ANYTHING, IT CAN BE FATAL, AND MOST KIDS DON'T SURVIVE THE AGE OF ONE.

SO TO HEAR HER STORY AND THE SAGA OF DR. KOHN AND HIS TEAM AT UCLA AND WHAT THEY'VE BEEN ABLE TO DO, NOT JUST FOR EVIE, BUT FOR, AS YOU HEARD, I THINK IT WAS 40 SOME ODD PEOPLE THEY'VE TREATED NOW,

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ALL OF WHICH ARE DOING GREAT. IT'S WHAT WE'RE ALL ABOUT HERE. IT WAS JUST WONDERFUL TO HEAR THE STORY AND JUST SUGGESTIVE OF HOW THE TEAM EFFORT AND WHAT WE'RE DOING IS WORKING. IT'S FANTASTIC.

THE BOARD, I WILL JUST SAY, IS PROUD OF THE INVESTMENT WE'VE MADE TO SUPPORT THIS REWARDING BRIDGES PROGRAM. YOU TALK TO THESE STUDENTS, AND THEY ARE SO GRATEFUL TO HAVE ACCESS TO THE STEM CELL WORK THAT THEY ARE ABLE TO ENGAGE IN WHICH THEY COULDN'T BUT FOR GRANTS, AND ARE UNDOUBTEDLY ENTHUSIASTICALLY PART OF THE PIPELINE OF GREAT TALENT THAT WE HAVE THAT'S GOING TO STEP INTO THE INCREASING STEM CELL WORK BEING DONE IN THE STATE.

SECOND ONE I'D LIKE TO TALK ABOUT, WHICH IS, AGAIN, A PERSONAL FAVORITE, I THINK, OF EVERYBODY ASSOCIATED IS THE SPARK PROGRAM. THIS IS THE HIGH SCHOOL KIDS WHO ARE -- THIS PROGRAM IS REMARKABLE BECAUSE THESE KIDS, THEY APPLY FOR THESE THINGS AND THEY GO INTO THE PROGRAM, AND THEY MAY HAVE HAD SOME DEALINGS IN STEM CELLS IN THEIR HIGH SCHOOL BIOLOGY CLASSES, BUT THERE'S A LOT OF STUFF TO COVER AND THAT'S NOT A BIG PART OF THE COURSE, AND SO THEY'RE REALLY STARTING OUT KIND OF NEW.

AND THEY GO FOR A NUMBER OF WEEKS EVERY SUMMER, AND IT ENDS IN A CONFERENCE THAT WE CONVENE

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WHERE THEY PRESENT POSTERS ON THEIR WORK. AND IT IS UNBELIEVABLE BECAUSE THESE KIDS GO FROM HAVING SOME FUNDAMENTALS TO SOUNDING LIKE PH.D. STUDENTS AS THEY SIT OUTLINING THEIR WORK ON THE POSTER. AND THE LEVEL OF ENTHUSIASM THAT THEY BRING AND THE LEVEL OF INTEREST AND THE CLEAR INDICATION THAT THIS IS SOMETHING THEY WANT TO PURSUE DOWN THE ROAD IS NOT ONLY GREAT TO HEAR, BUT IT REALLY VALIDATES CIRM'S THINKING THAT IT WOULD BE HELPFUL TO TRY TO FACILITATE THESE KINDS OF THINGS.

SO JUST A COUPLE OF STATS ON THAT. THE SECOND ANNUAL SPARK CONFERENCE, WHICH PREVIOUSLY, BY THE WAY, WERE THE CREATIVITY AWARDS, HOSTED IN EARLY AUGUST AT THE CITY OF HOPE, DR. MALKAS. SEVEN SPARK PROGRAMS WERE IN ATTENDANCE: CALTECH, CEDARS, CITY OF HOPE, UC DAVIS, UCSF, STANFORD, CHILDREN'S HOSPITAL OAKLAND RESEARCH INSTITUTE. TOTAL OF 56 KIDS PRESENTED THEIR RESEARCH AND THEIR POSTER, AND RESEARCH PRESENTATIONS, AS ALWAYS, WERE OFF THE CHARTS.

THE CONFERENCE FEATURED A KEYNOTE ADDRESS FROM CLIVE SVENDSEN AT CEDARS. ANOTHER HIGHLIGHT WAS A SPECIAL TALK BY ALS PATIENT ADVOCATE LANCE MCCOLLOUGH. HE'S ASSISTANT VARSITY COACH OF THE BAKERSFIELD HIGH SCHOOL FOOTBALL TEAM. LANCE HAS

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ALS. AND INSTEAD OF LETTING THE DISEASE TAKE OVER HIS LIFE, HE'S BECOME A MOTIVATIONAL SPEAKER TO OTHER ALS PATIENTS AND YOUNG ATHLETES. OTHER SPEAKERS INCLUDED FORMER BRIDGES AND SPARK ALUMNI FROM THE CITY OF HOPE AND AN AFTERNOON KEYNOTE ADDRESS BY DR. JOHN ZAIA.

FEW OTHER ASSORTED SPARK FACTS. IT STARTED 2016. IT'S A PROGRAM TO TRAIN THE NEXT GENERATION OF STEM CELL SCIENTISTS AND HAVING STUDENTS FOCUS ON PATIENT ENGAGEMENT ACTIVITIES AND SCIENCE COMMUNICATION. HUNDRED FOURTEEN SPARKS STUDENTS HAVE GONE THROUGH OUR PROGRAM IN THE FIRST TWO YEARS.

OVERALL FEEDBACK FROM SPARK AND ITS PREDECESSOR CREATIVITY PROGRAMS PAST ALUMNI FROM THIS PROGRAM HAVE BEEN SHOWN TO PURSUE CAREERS IN THE SCIENCES IN SIGNIFICANT PERCENTAGES; THUS, MANY OF THESE STUDENTS WILL LIKELY CONTINUE WORKING IN STEM CELL RESEARCH IN THEIR UNDERGRADUATE YEARS AND BEYOND. SPARK ALUMNI ARE ATTENDING TOP UNIVERSITIES AND COLLEGES, INCLUDING THE UC'S, BERKELEY, UCLA, AND DAVIS, STANFORD, YALE, ETC., TO PURSUE DEGREES IN SCIENCE AND MEDICINE.

I WANT TO JUST SINGLE OUT. TODD, CAN YOU HEAR ME OUT THERE? I WANT TO SINGLE OUT TODD FOR

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HIS LEADERSHIP IN THE BRIDGES PROGRAM. HE HEADED UP THE EFFORT HERE FOR CIRM, DID A TERRIFIC JOB. AND KAREN FOR DOING LIKEWISE IN THE SPARK PROGRAM. YOU GUYS DO A TERRIFIC JOB, AND IT MAKES US -- EVERY YEAR IT BRINGS BIG SMILES TO OUR FACE TO SEE THESE KIDS AND KNOW WHAT THEY ARE ALL ABOUT AND INTERESTED IN DOING.

SO WITH THAT, I WANT TO JUST ASK THE BOARD IF THERE ARE ANY COMMENTS ON ANY OTHER ITEMS ANYBODY CARES TO SPEAK ABOUT? I THINK WE'VE GOTTEN THROUGH A LOT OF VERY IMPORTANT MATERIAL TODAY. I'D LIKE TO SAY, ONCE AGAIN, TO CIRCLE BACK TO THE BEGINNING, MARIA, CONGRATULATIONS. YOU ARE IN EVERYBODY'S ESTIMATION THE RIGHT PERSON AT THE RIGHT TIME TO CARRY CIRM FROM HERE ON INTO THE FUTURE. SO MANY CONGRATULATIONS.

(APPLAUSE.)

DR. MILLAN: THANK YOU VERY MUCH AGAIN.

CHAIRMAN THOMAS: LASTLY, I'D LIKE TO THANK -- I GOT TO TELL YOU, I DON'T KNOW WHAT ANYONE ELSE THINKS, BUT I THINK HAVING THE BOARD MEETINGS HERE WORKS OUT GREAT. THE SPACE IS GREAT. THE ROOM IS THE RIGHT SIZE. SPECTACULAR VIEWS. AND I WANT TO GIVE A MAJOR SHOUT OUT TO MEMBERS OF THE TEAM WHO PUT THIS ALTOGETHER, NOT JUST THE ROOM AND THE

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MEETING, BUT THE FIVE-STAR FARE WE HAD FOR LUNCH. SORRY FOR YOU GUYS ON THE PHONE WHO WEREN'T ABLE TO HAVE IT. IT WAS REALLY TASTY. TO AMY AND KIM AND DOUG AND ILLIANA AND TRICIA AND EVERYBODY WHO CONTRIBUTED HERE TO THIS EFFORT, THANK YOU SO MUCH FOR ALL YOUR WORK. IT RAN SEAMLESSLY, AND WE ARE VERY GRATEFUL. SO THANK YOU.

(APPLAUSE.)

CHAIRMAN THOMAS: SO THAT CONCLUDES TODAY'S AGENDA. THE NEXT IN-PERSON MEETING WILL BE IN DECEMBER. WE WILL HAVE OUR REGULAR MEETINGS OF THE BOARD AND THE APPLICATION REVIEW SUBCOMMITTEE IN OCTOBER AND NOVEMBER. AND WISH EVERYBODY A SAFE TRIP BACK HOME. AND THANKS TO EVERYBODY FOR COMING, AND WE WILL SEE YOU IN DECEMBER.

(THE MEETING WAS THEN CONCLUDED AT 1:47 P.M.)

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REPORTER'S CERTIFICATE

I, BETH C. DRAIN, A CERTIFIED SHORTHAND REPORTER IN AND FOR THE STATE OF CALIFORNIA, HEREBY CERTIFY THAT THE FOREGOING TRANSCRIPT OF THE PROCEEDINGS BEFORE THE INDEPENDENT CITIZEN'S OVERSIGHT COMMITTEE AND THE APPLICATION REVIEW SUBCOMMITTEE OF THE CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE IN THE MATTER OF ITS REGULAR MEETING HELD AT THE LOCATION INDICATED BELOW

CALIFORNIA INSTITUTE FOR REGENERATIVE MEDICINE
1999 HARRISON STREET, SUITE 1650
OAKLAND, CALIFORNIA
ON
SEPTEMBER 28, 2017

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

BETH C. DRAIN, CA CSR 7152
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